Q2 2021 Results

Investor presentation

NOVARTIS | Reimagining Medicine

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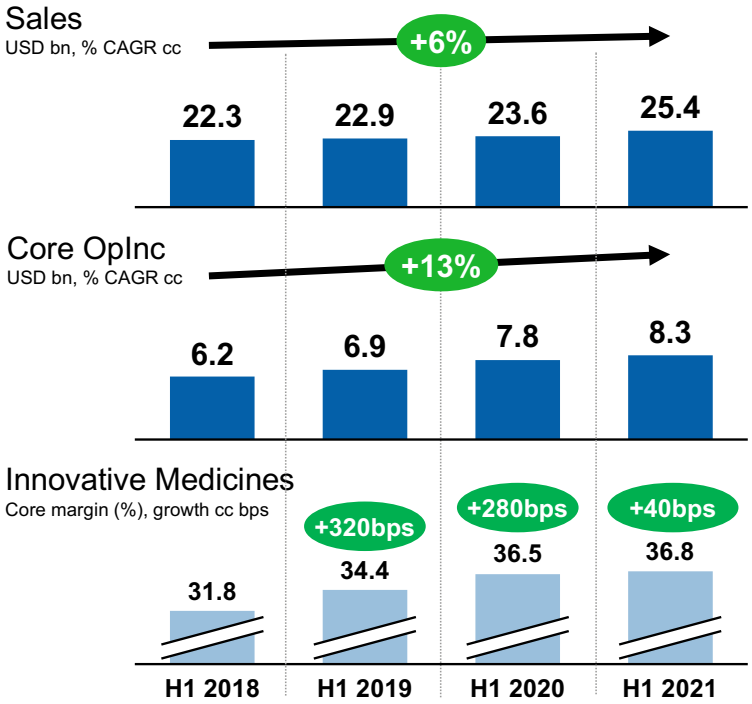
Chief Executive Officer

Company overview

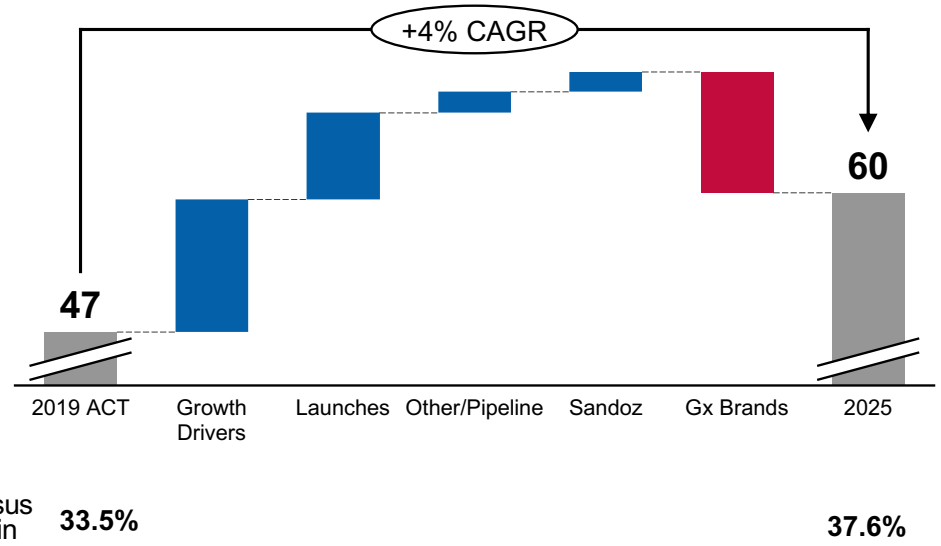
Consistent long-term performance driving confidence for the future

Consistent strong performance



Confident on growth outlook

External expectations based on analyst consensus, as presented at November 2020 Meet Novartis Management[1] (USD bn)



All growth % in cc. IM – Innovative Medicines division. Constant currencies (cc), core results are non-IFRS measures; explanation can be found on page 48 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY. 1. Source: Novartis Investor Relations in-house consensus as of November 12, 2020.

Strong Q2 performance across our value drivers

Growth[1] 1

Q2 Group sales **+9%**; H1 +3%

Q2 IM sales **+10%**; H1 +5%

Q2 Sandoz sales **+5%**; H1 -5%

Productivity[1] 2

Q2 Group Core operating income **+13%**; H1 +2%

Q2 IM core operating income **+14%**; H1 +6%

Q2 IM core margin 37.3% (**+1.3%**pts cc); H1 36.8%

Innovation 3

Iptacopan	Ph2 studies met endpoints in PNH, IgAN, C3G (IA); Ph3 enrolling
^{177}Lu-PSMA-617	Reduced mortality in patients with mCRPC; received FDA BTD
Asciminib	Submitted in US and EU for 3L CML
Kymriah®	ELARA pivotal study positive final readout in FL enabling submission
Leqvio®	Resubmitted new drug application to FDA (manufacturing CRL)
Zolgensma®	Showed transformative efficacy in presymptomatic SMA

ESG 4

Delivered 1bn antimalarial courses to patients in need since 1999

Advancing efforts on clinical trial diversity

10-year commitment to address root causes of health disparities

All growth % in cc IM – Innovative Medicines division BTD – Breakthrough Therapy designation 1. Constant currencies (cc), core results are non-IFRS measures; explanation can be found on page 48 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Key growth drivers and launches continued momentum in Q2

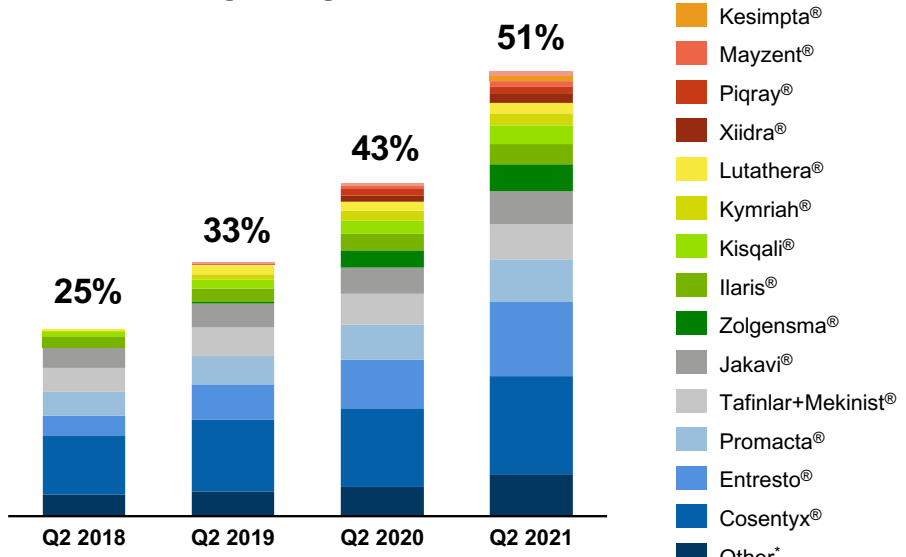
Q2 key growth driver sales momentum[1]

	Sales USD Million	Growth vs. PY USD Million	Growth vs. PY cc
Entresto	886	306	46%
Cosentyx	1,175	231	21%
zolgensma	315	110	48%
PROMACTA (eltrombopag)	513	91	18%
JAKAVI ruxolitinib	398	88	19%
Xolair omalizumab	355	66	14%
KISQALI ribociclib	225	66	36%
Kesimpta (ofatumumab)	66	66	nm
Tafinlar + Mekinist	425	54	10%
ILARIS (canakinumab)	247	47	21%
xiidra	118	39	48%
MAYZENT (siponimod) tablets	69	35	96%

nm – not meaningful

Driving portfolio rejuvenation

Key growth drivers and launches
51% of IM sales, growing 29% in Q2



*Includes Xolair®, Beovu®, Adakveo®, Luxturna®, Tabrecta®, Enerzair®, Atectura® and Leqvio®

1. Innovative Medicines division. Constant currencies (cc) is a non-IFRS measure; explanation of non-IFRS measures can be found on page 48 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY

Sandoz business stabilizing in Q2

Sandoz performance starting to stabilize

(vs. PY, in % cc)

Net total sales
Q2 2021: **+5%**
H1 2021: -5%

Biopharma sales
Q2 2021: **+5%**
H1 2021: +6%

Core operating income
Q2 2021: **+3%**
H1 2021: -19%

Challenges remain due to COVID-19 related disruptions

Impact on Q2 growth

Sales growth excl. PY forward purchasing de-stocking[1]	**-1%** cc

COVID-19 impact mainly Retail Generics

Historically weak cough and cold season, decreased Anti-Infectives

Confidence for future: investments in biosimilars and select Retail generics

Biosimilars	**Pipeline doubled** in ~3 years **Strategic focus**: oncology, immunology, endocrinology, underserved disease areas
Generics	Strengthening antibiotics manufacturing setup Select investments in complex areas, incl. oncology solids, respiratory, injectables

1. Growth excl. PY forward purchasing de-stocking is a non-IFRS measure; explanation can be found on page 61 of Condensed Interim Financial Report

Broad pipeline of novel medicines continued to progress in Q2

Approvals

Cosentyx®	US: pediatric PsO
Entresto®	China: essential hypertension

Submissions

LEQVIO®	US: resubmission
ABL001 asciminib	US, EU: chronic myeloid leukemia, 3L
Cosentyx®	US, EU: juvenile idiopathic arthritis

Designations

AAA617 ^{177}Lu-PSMA-617	FDA Breakthrough Therapy designation in mCRPC
MBG453 sabatolimab	FDA Fast Track designation in myelodysplastic syndrome

Readouts and publications (selected)

●	**Iptacopan**	Ph2 – PNH, IgAN, C3G (IA)
●	**Kymriah®**	Ph2 (pivotal) – r/r FL (ELARA)
●	**Zolgensma®**	Ph3 – SMA (SPR1NT and STR1VE)
●	**Alpelisib**	Ph3 – PROS (EPIK-P1)
●	**Tislelizumab**	Ph3 – 2L ESCC (RATIONALE 302)
●	**Beovu®**	Ph3 – nAMD (MERLIN)

● Positive
● Negative

See last slide for all abbreviations

Moving forward a breadth of assets to drive long-term growth

Selected opportunities, **expected 2021 milestones** and additional indications

Lifecycle management

Entresto®	China approval for essential hypertension
Cosentyx®	HS: SUNRISE, SUNSHINE **Ph3 readout H2 2021**
	L. Planus, jPsA/ERA (submitted), GCA, lupus nephritis
Kisqali®	aBC: MONALEESA-2 OS **readout H2 2021**
	HR+/HER2- BC (adj) **readout 2022**
Leqvio®	Hyperlipidemia: **resubmitted to FDA**
	CVRR-LDLC
Zolgensma®	SMA IT

Pharmaceuticals

Iptacopan (LNP023)	IgAN, PNH, C3G, aHUS: **Ph3 start 2021**
	iMN
Iscalimab (CFZ533)	Sjögren's, kidney Tx, liver Tx
Ligelizumab (QGE031)	CSU: PEARL 1, 2 **Ph3 readout H2 2021**[1]
	CINDU, food allergy **Ph3 start H2 2021**
Pelacarsen (TQJ230)	CVRR-Lp(a)
Branaplam (LMI070)	HD: **Ph2b start H2 2021**

Oncology

Canakinumab (ACZ885)	NSCLC 1L: CANOPY-1 **Ph3 readout H2 2021**
	NSCLC adjuvant
^{177}Lu-PSMA-617	mCRPC 3L: VISION **positive readout; submission H2 2021**
	mCRPC pre-taxane, mHSPC: **Ph3s started**
Sabatolimab (MBG453)	HR-MDS: STIMULUS **Ph2 continues blinded after CR readout**[2]
	AML
TNO155 combinations	Solid tumors, multiple combinations being explored in ongoing trials
Tislelizumab (VDT482)	2L esophageal cancer and NSCLC: **submission 2021**

'Wild Cards'

Asciminib (CML 1L: **Ph3 start H2 2021**), LNA043 (Osteoarthritis: **Ph2b started H1 2021**), CSJ117 (Asthma), QBW251 (COPD), LXH254 (BRAF/NRASm melanoma, mRAS/RAF NSCLC), NIS793 (Solid tumors, **mPDAC Ph3 start H2 2021**)

1. Q4/2021-Q1/2022 potential COVID-19 impact. 2. Planned DMC readout for CR completed, study continues blinded to PFS readout, with submission in 2022/2023 using PFS and/or OS outcomes of Ph2 and/or Ph3 trial.

Inclisiran resubmission of NDA filed with FDA – PDUFA action date Jan 1, 2022

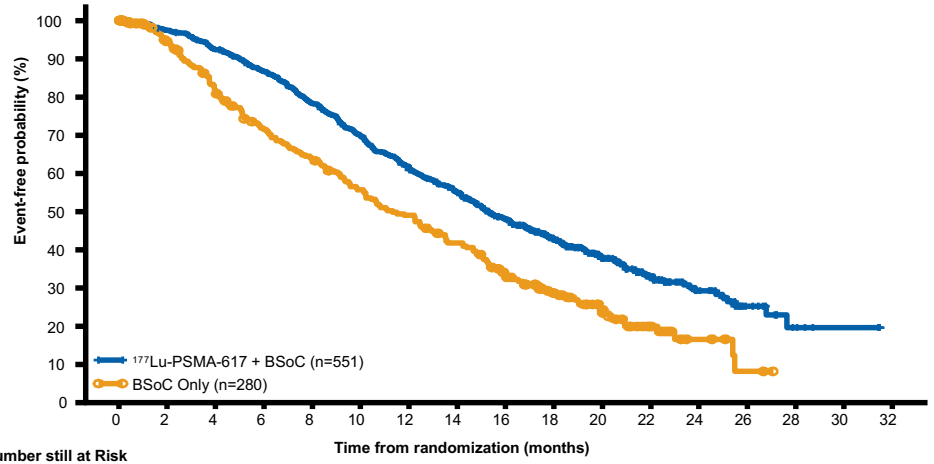
- Own site in Schaftenau (Austria) listed as the manufacturing location for finished product within resubmission
- Resubmission addresses the FDA Complete Response Letter (CRL) issued in December 2020, stating unresolved facility inspection-related conditions at a third-party manufacturing facility
- FDA did not raise any concerns related to the efficacy or safety of inclisiran
- The transfer of the manufacturing of inclisiran (finished product) to the Novartis-owned facility at Schaftenau was planned and initiated in 2020, prior to the receipt of the CRL
- Class 2 Resubmission, PDUFA action date Jan 1, 2022

^{177}Lu-PSMA-617 reduced risk of death by 38%, and radiographic progression or death by 60% in patients with mCRPC (VISION)

Presented at ASCO 2021

OS HR[1]: 0.62 (95%CI: 0.52, 0.74)

Median OS: 15.3 months (14.2, 16.9)[3] vs. **11.3** (9.8, 13.5)[3]

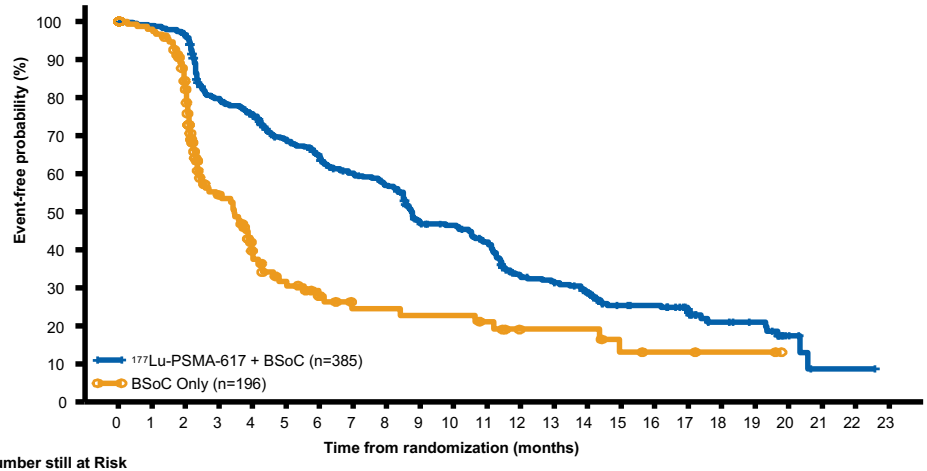


Number still at Risk

^{177}Lu-PSMA-617 + BSoC	551	535	506	470	425	377	332	289	236	166	112	63	36	15	5	2	0
BSoC Only	280	238	203	173	155	133	117	98	73	51	33	16	6	2	0	0	0

rPFS HR[1]: 0.40 (99.2%CI: 0.29, 0.57)

Median rPFS: 8.7 months (7.9, 10.8)[2] vs. **3.4** (2.4, 4.0)[2]



Number still at Risk

^{177}Lu-PSMA-617 + BSoC	385	373	362	292	272	235	215	194	182	146	137	121	88	83	71	51	49	37	21	18	6	1	1	0
BSoC Only	196	146	119	58	36	26	19	14	14	13	13	11	7	7	7	4	3	3	2	2	0	0	0	0

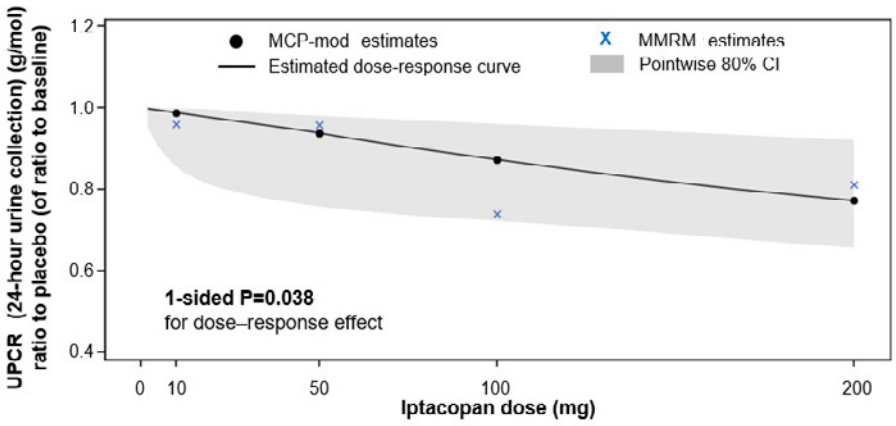
- **Regulatory submissions** in US and EU on track for **H2 2021**
- Data support investigating ^{177}Lu-PSMA-617 in **earlier lines of therapy**
- Two Ph3 studies in pre-taxane 1L / 2L mCRPC **PSMAfore** and mHSPC **PSMAddition** already underway

1. p<0.001, stratified log-rank test 1-sided. 2. 99.2% CI, in line with hypothesis testing strategy. 3. 95% CI.

Iptacopan shows clinically meaningful reduction in proteinuria and stabilization of renal function in patients with IgAN and C3G

Primary endpoint data presented at ERA-EDTA 2021

IgAN

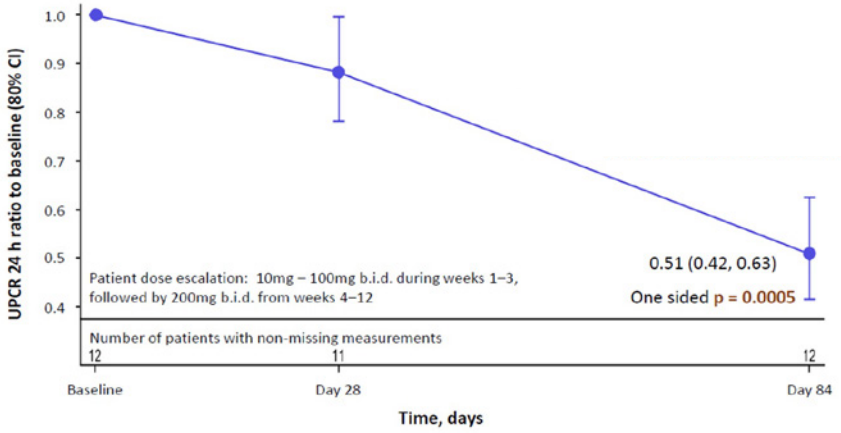


- **23% reduction in proteinuria at 3 months** (200mg BID)
- Encouraging trend to **early stabilization of renal function** (eGFR)
- Well tolerated; no serious infections
- **Ph3 APPLAUSE-IgAN:** Ongoing to support iptacopan filings worldwide

IA data on primary endpoint presented at ASN 2020

C3G

EU PRIME designation

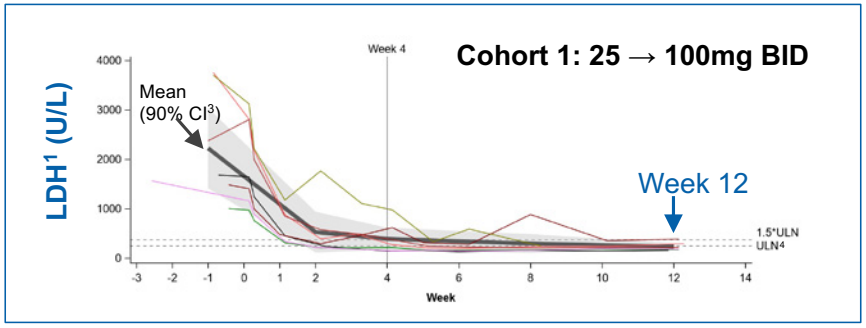


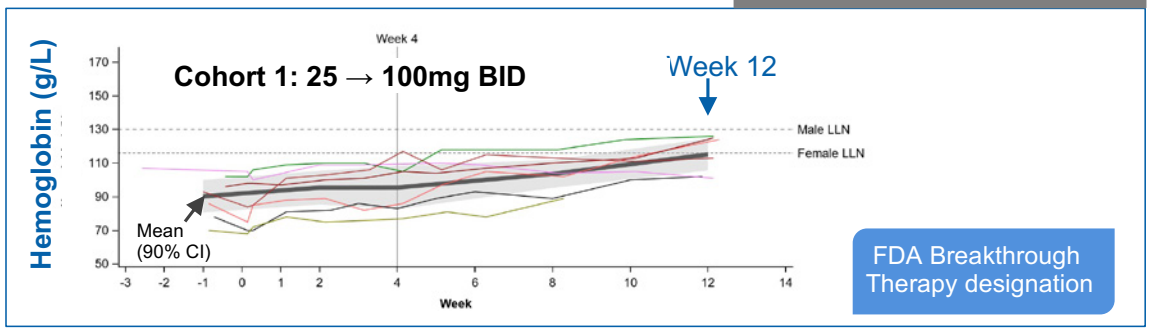
- **49% reduction in proteinuria at 3 months**
- **Stabilization of renal function** (eGFR) at 3 months
- Well tolerated with no unexpected or new safety findings
- Final Ph2 readout imminent
- **Ph3 APPEAR-C3G:** Enrolling, will support filings worldwide

BID – twice daily CI – confidence interval eGFR – estimated glomerular filtration rate MCP-mod – Multiple Comparison Procedure-Modelling MMRM – mixed model repeated measurements UPCR – Urine protein to creatinine ratio

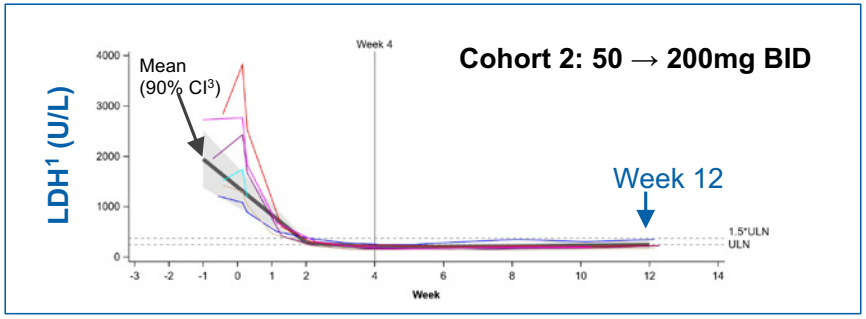
Iptacopan reduces LDH and increases hemoglobin in PNH

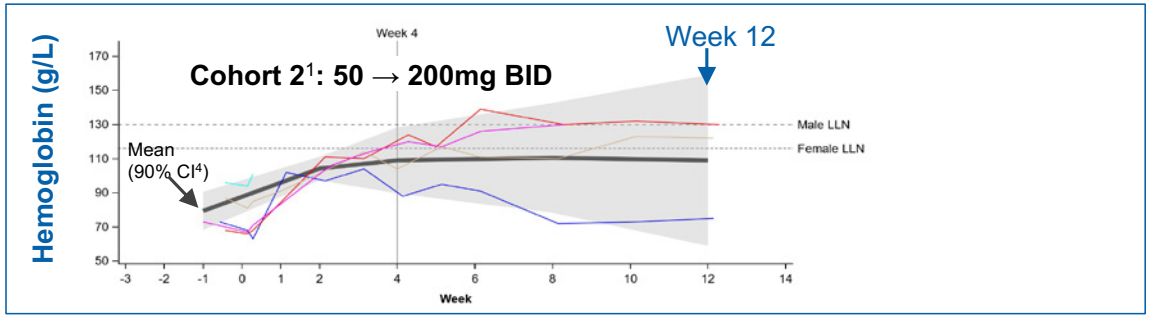
New data (anti-C5 naive) presented at EHA 2021





FDA Breakthrough Therapy designation





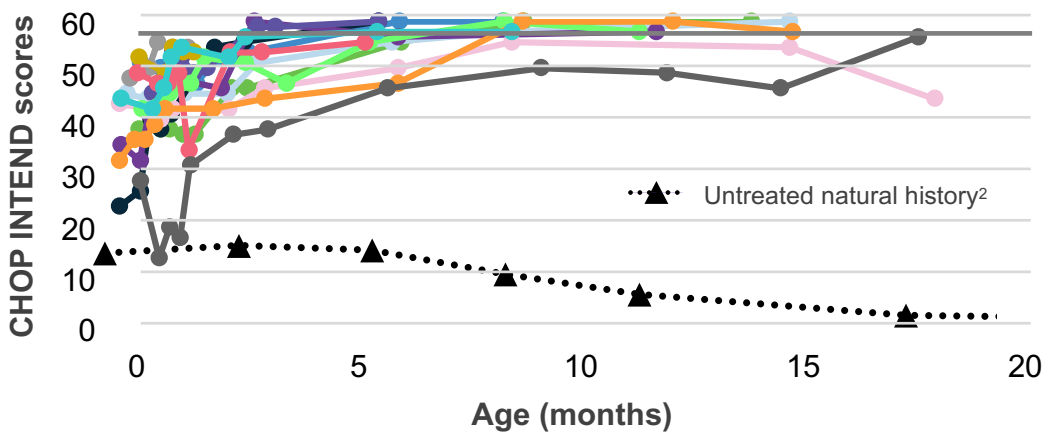
- New data (EHA 2021) shows **clinically important benefits of monotherapy iptacopan** in anti-C5 treatment naive PNH patients
- Previous Ph2 shows **iptacopan** provided clinical **benefits as add-on to eculizumab** in PNH residual hemolysis (EBMT 2020)
- **Ph3 APPLY-PNH** study to assess **superiority of iptacopan vs. anti-C5** therapy ongoing, will support filings worldwide

LDH – Lactate dehydrogenase BID – Twice a day CI – Confidence interval ULN – Upper limit of normal 1. One patient in Cohort 2 was excluded for Hb analyses due to RBC transfusion that occurred between screening and baseline, raising Hb from 71 to 110 g/L. Source: Jang JH, et al. Iptacopan Effectively Controls Intra- And Extravascular Hemolysis And Leads To Durable Hemoglobin Increase In Patients With Treatment-Naïve PNH.

Zolgensma® SPR1NT data (2-copy cohort) demonstrate transformative, age-appropriate development when used presymptomatically

n=14 (2 copy cohort)

Achieved CHOP INTEND[1] score ≥58 points **100%**



Mean age at dosing: 20.6 days (8-34 days);
No serious, treatment-related adverse events

Presented at EAN 2021

100% Met primary endpoint:
Sitting independently for ≥30 seconds[3]
Nearly all patients (11/14) within the WHO window for normal development

100% Met secondary endpoint:
Survival without permanent ventilation[4]

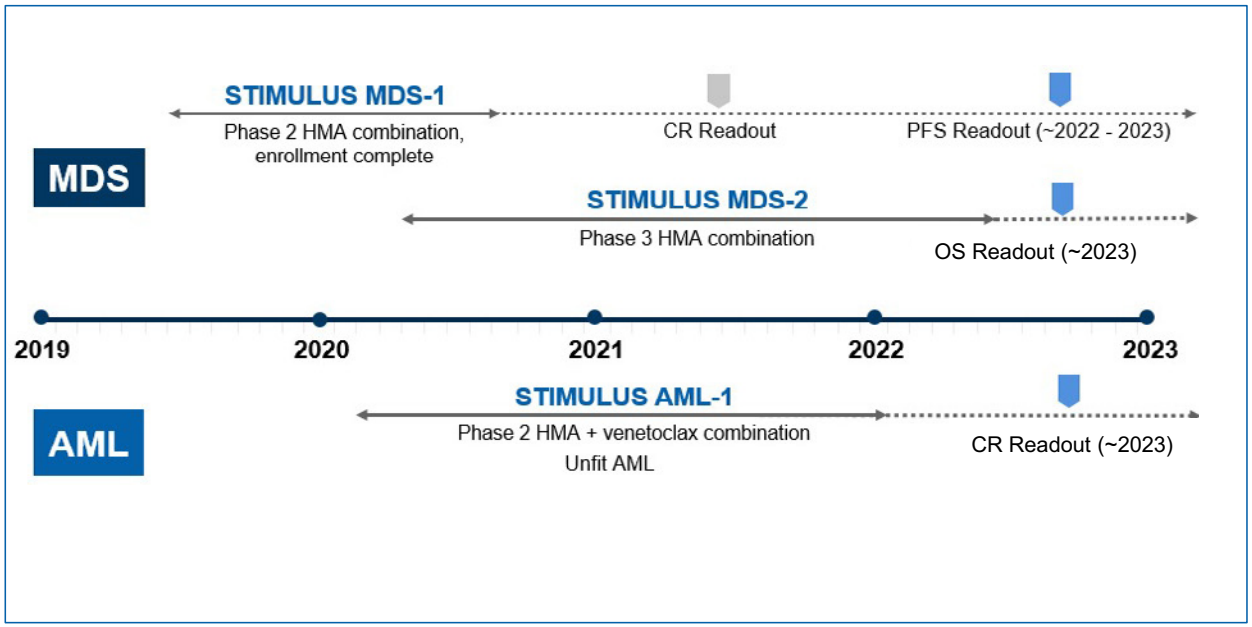
79% Standing alone for ≥3 seconds (Bayley)
Most (7/11) within WHO window

64% Walking alone (Bayley)
Majority (5/9) within WHO window

Data reinforces Zolgensma® as foundational therapy for both presymptomatic and symptomatic children with SMA

1. CHOP INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders. 2. Natural history data from NeuroNEXT prospective natural history study in SMA infants with two copies of *SMN2*. 3. Functional independent sitting for ≥30 seconds (Bayley-III item #26) at any visit up to 18 months of age. 4. Survival at age 14 months.

Sabatolimab: STIMULUS program progressing in MDS and AML



- Ph1 sabatolimab + HMA data showed promising and durable response rates (58% ORR in MDS[1])
- **STIMULUS MDS-1: Ph2** randomized, double-blind, 2 primary endpoints: CR, PFS
  In June 2021, the **DMC determined** that the study **should continue blinded** until PFS readout (event-driven)
- **STIMULUS MDS-2: Ph3** randomized, double-blind, primary endpoint: OS (event-driven)
  Enrollment ahead of target

- Parallel execution of trials offers a range of filing options between 2022 and 2023 depending on PFS and/or OS outcomes
- STIMULUS program has expanded with additional trials in AML and MDS including low-risk MDS

AML – Acute Myeloid Leukemia MDS – Myelodysplastic Syndrome CR – Complete Remission PFS – Progression-free Survival ORR – Overall response rate OS – Overall Survival DMC – Data Monitoring Committee

1. Wei A et al., EHA, June 2021.

Strong progress on ESG in Q2, with focus on new health equity initiatives and advancing our Global Health efforts

Leading on health equity

New target on clinical trial diversity

Embedding diversity & inclusion in 100% of Ph3 studies with US participation

Pledged 10-year commitment[1]

USD 20m to empower 1200 African American students, USD 13.7m to establish research centers

Addressing unmet need in breast cancer

Multiyear commitment to address racial disparities in breast cancer: estimated at USD 93bn in excess medical care costs in US

Advancing our patient reach

Malaria milestone

1bn courses of antimalarial treatment delivered since 1999

Accelerate use of digital technologies for global health

First use case for dengue which affects 400m cases / year
Developing disease surveillance solution with HP Enterprises

1. Via the Novartis US Foundation in collaboration with Historically Black Colleges, Universities, other organization and companies.

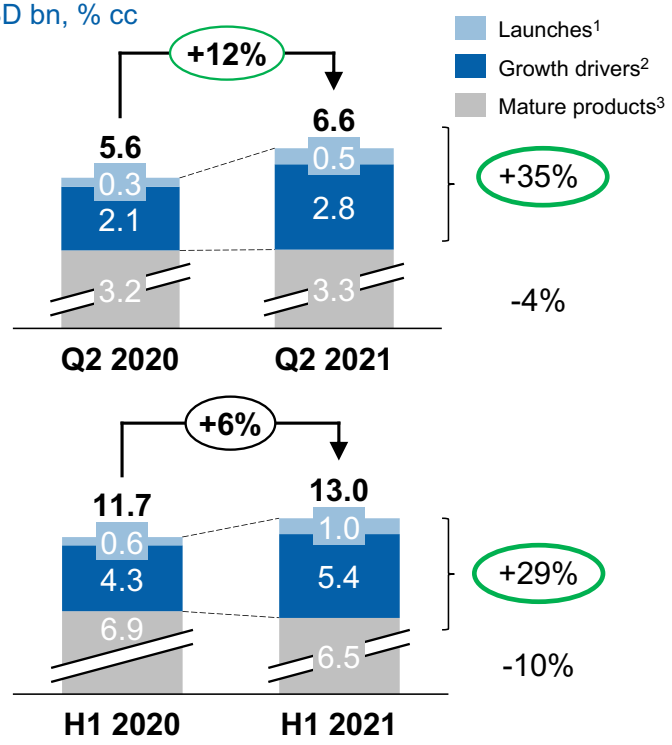
Marie-France Tschudin

President, Novartis Pharmaceuticals

Pharmaceuticals grew +12% in Q2 with growth drivers and launches showing strong momentum

Pharmaceuticals net sales

USD bn, % cc



Growth drivers showing strong momentum in Q2 vs. prior year

- Cosentyx® and Entresto® together reached USD >2bn
- Zolgensma® up +48% YoY driven by geographic expansion
- Ilaris® up +21% driven by Adult Onset Still Disease, Periodic Fever Syndrome

New portfolio building foundation for future growth

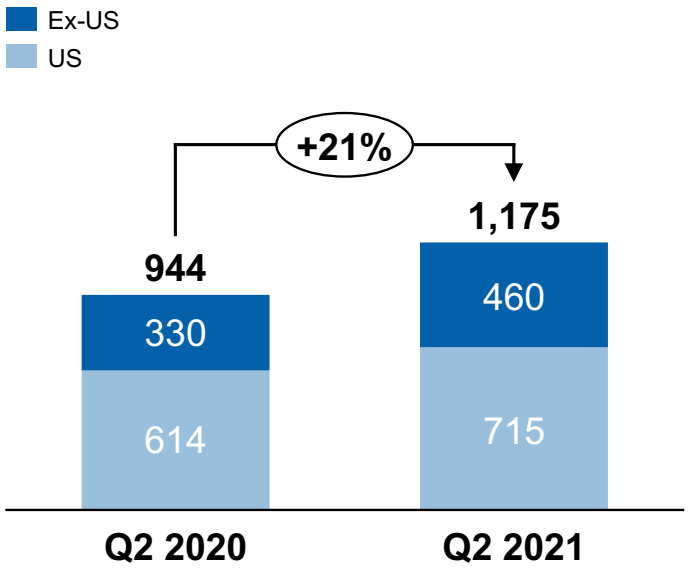
- Growth drivers and launches represent 51% of sales (up from 43% Q2 2020)
- Cosentyx® LCM progressing with regulatory milestones for pediatric portfolio
- Leqvio® re-submitted in US – launch preparations on track

LCM – Lifecycle Management. All % growth relate to cc unless otherwise stated. 1. Zolgensma®, Kesimpta®, Mayzent®, Beovu® , Luxturna®, Leqvio®, Enerzair® and Atectura®. 2. Cosentyx®, Entresto®, Xolair® , Ilaris® , Xiidra® and Aimovig® 3. All other brands.

Cosentyx® grew 21% in Q2, momentum expected to continue through 2021

Sales evolution

USD m, % cc



Double-digit growth expected to continue in H2

- US: growing volume in line with market across indications (vs. Q1)
- EU: leading biologic in PsO, leading originator biologic in SpA[1]
- Growth continues in other markets including China post-NRDL

LCM continues to reinforce differentiated efficacy and safety

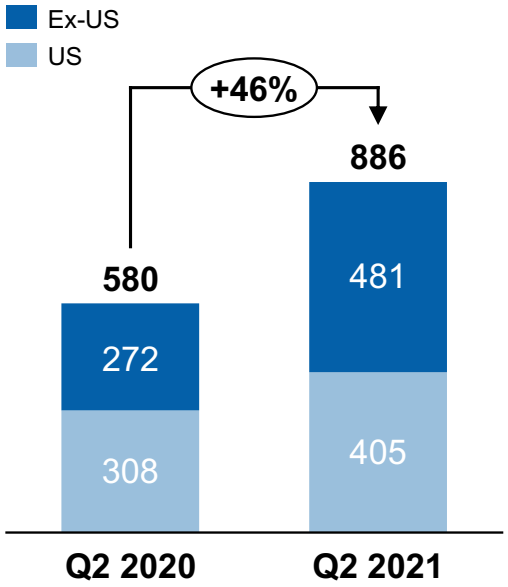
- US Ped PsO approved
- EU / US submitted for jPsA & ERA
- Ph3 readout for Hidradenitis Suppurativa on track for H2 2021
- US 300mg autoinjector approval expected H2 2021
- PsO dose flexibility approval expected H2 2021 EU / H1 2022 US

NRDL – National Reimbursement Drug List Ped PsO – Pediatric Psoriasis PsO – Psoriasis SpA - Spondyloarthritis jPsA – Juvenile psoriasis arthritis ERA – Enthesitis related rheumatoid arthritis 1. TRx - EU data March 2021.

Entresto® grew 46% in Q2; confident in future growth based on 1L guideline position, label update and geographic expansion

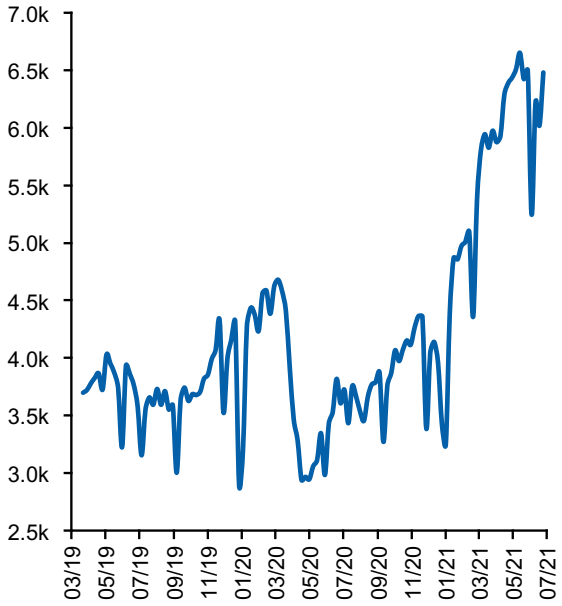
Sales evolution

USD m, % cc



Weekly NBRx[1]

New-to-brand prescriptions



Strong momentum worldwide

- US: sales grew +31% to USD 405m
- Ex-US: sales up +63% to USD 481m
- China now 2nd biggest market, accounting for ~ one fourth of ex-US sales

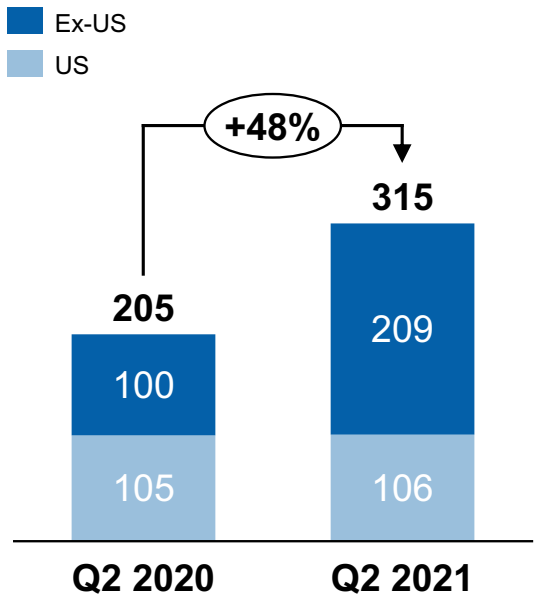
Confidence in future growth trajectory

- **ACC ECDP**[2] and **draft ESC guidelines**[3] recommend ARNI first-line for all appropriate HFrEF patients, including *de novo* initiation
- **Label expansion** strengthens essential role of Entresto® across HF continuum
- China approval in **essential hypertension**

See slide 55 for references. NBRx – New-to-brand Prescriptions ACC – American College of Cardiology ECDP – Expert Consensus Decision Pathway ESC – European Society of Cardiology ARNI – Angiotensin Receptor Neprilysin Inhibitor HFrEF – Heart Failure with reduced Ejection Fraction HF – Heart Failure All % growth relate to cc unless otherwise stated.

Zolgensma® grew 48% in Q2, potentially transformative efficacy in pre-symptomatic SMA

Sales evolution
USD m



Q2 highlights

- Continued strong sales growth with expanding access in Europe and emerging markets
- Stable US business, driven by incident patients
- Approval in 41 countries; access pathways in 19 countries
- 1.4k+ patients have been treated with Zolgensma® worldwide[1]

Future growth drivers

- Reimbursement: Implementation of H1 milestones (e.g. NHS)[2] and global expansion
- Newborn screening: met target in US of >80%, on track for 20% in EU by end 2021

New data reinforce strong clinical benefit[3]

- Age-appropriate development when used presymptomatically (SPR1NT) and consistent, significant benefit in symptomatic children (STR1VE-EU)
- Reinforces Zolgensma® as **foundational therapy for both presymptomatic and symptomatic children with SMA**

1. Commercially, via managed access programs and in clinical trials. 2. NHS: National Health Service in England. 3. Presented at European Academy of Neurology (EAN) 2021.

Kesimpta® launch momentum continues – demand expected to double in H2 vs. H1

Launch progress in US

NBRx +71%, **2nd** highest NBRx share ahead of Aubagio® and Tecfidera®

USD 66m Q2 sales, **+58%** QoQ[1]

>5k patients treated, **2x** vs. Q1

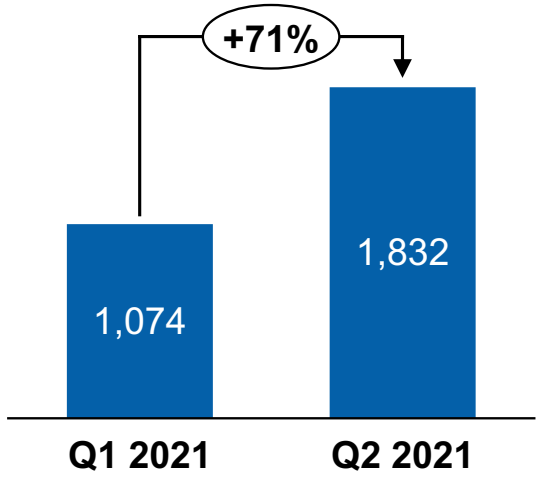
51% of patients naive or first switch

<5 days to 1st dose in 80% of patients

>500 new prescribers vs. Q1

Kesimpta® NBRx[2]

New-to-brand prescriptions



Foundation for continued growth

Dynamic market recovery as vaccination campaign progresses

Expansion of B-cell market as shift to high-efficacy therapies continues

Differentiation based on unique PIRA and IgG data

Growing awareness and familiarity with Kesimpta® to drive broader adoption

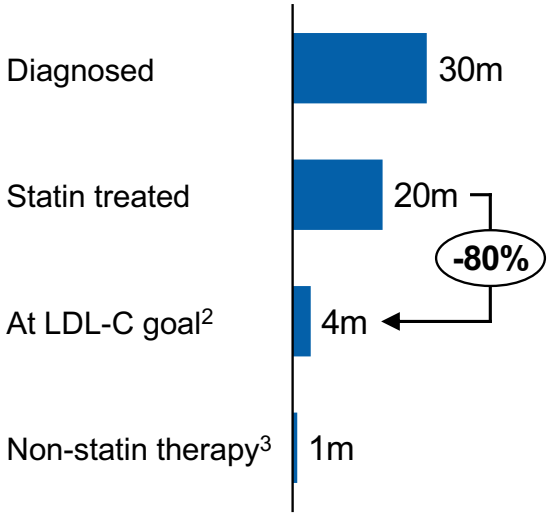
Launch in 13 markets expected by year end 2021

PIRA – Progression independent of relapses IgG – Immunoglobulin G 1. Excluding 9m adjustment related to faster than expected conversion from free to paid product in prior quarter. 2. Cumulative NBRx for the quarter.

Leqvio® – resubmission of NDA filed with FDA; preparing to launch innovative models to address access, adherence, affordability

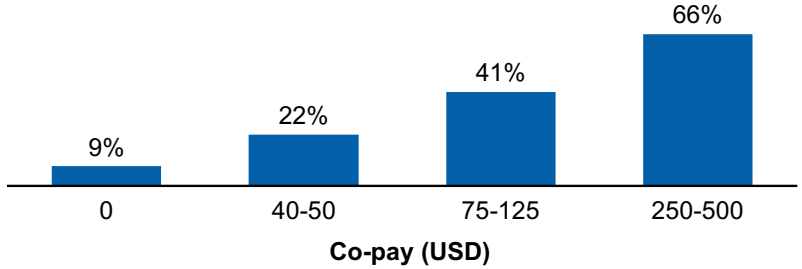
80% of patients not at goal

US ASCVD population[1]



Addressing access, adherence, affordability

PCSK9i abandonment by OOP cost[4]



Anticipated payer mix & co-pay for Leqvio® at launch[5]

Medicare Part B (39%)	80% pay as little as **0 USD**
Medicare Advantage (19%)	**0-20%** co-insurance
Commercial (34%)	Eligible patients pay as little as **0 USD**
Other[6] (8%)	**<10 USD**

Progress since Q1

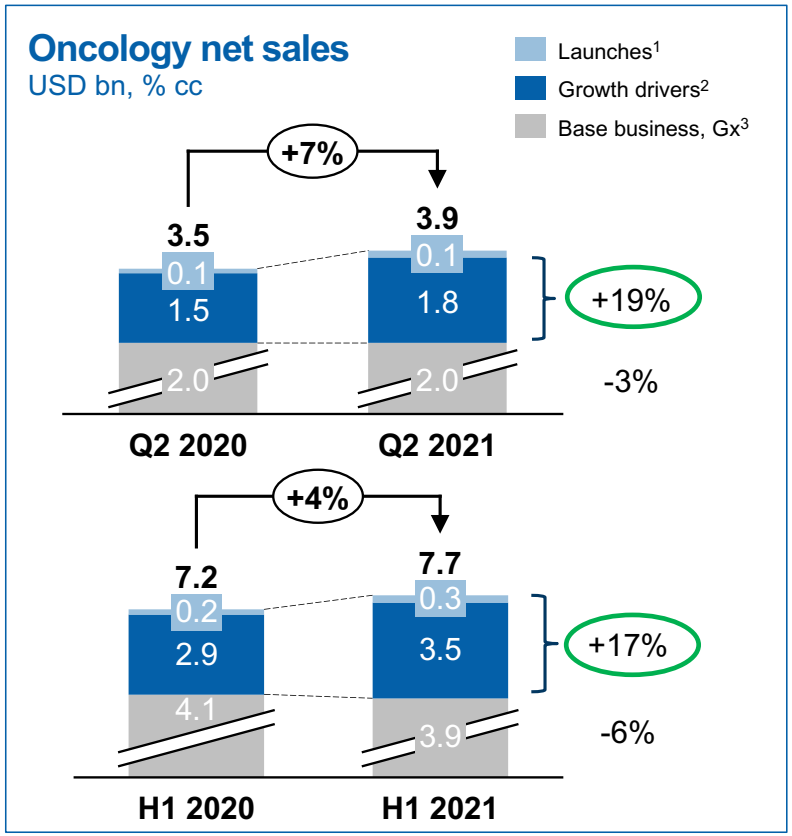
- ✓ **US:** NDA resubmission filed; PDUFA action date Jan 1, 2022
- ✓ **UK:** on track for launch Q3 2021
- ✓ **V-INITIATE** started to explore "Leqvio® first" strategy directly after statins[7]
- ✓ **V-INCEPTION** commenced to investigate Leqvio® initiation after recent ACS events[7]

See slide 55 for references. ASCVD – Atherosclerotic Cardiovascular Disease LDL-C – Low Density Lipoprotein Cholesterol ACS – Acute Coronary Syndrome

Susanne Schaffert

President, Novartis Oncology

Oncology grew 7% in Q2 despite continued COVID-19 impact



Solid Q2 performance fueled by growth drivers and recent launches

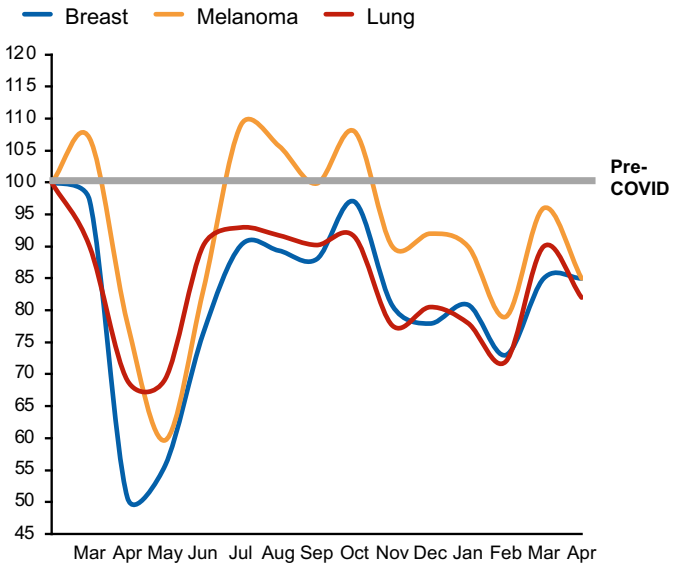
- Growth drivers and recent launches now constitute **50%** of sales (up from 45% Q2 2020)
- Key drivers:
  - Kisqali® Q2 sales USD 225m, +36%
  - Jakavi® Q2 sales USD 398m, +19%
  - Kymriah® Q2 sales USD 147m, +19%
  - Promacta®/Revolade® Q2 sales USD 513m, +18%
- COVID-19 continues to impact diagnosis and treatment rates in certain segments (e.g. hospital-initiated therapies and breast cancer)
- Ongoing Gx impact including Glivec®, Exjade®/Jadenu®, Afinitor®

1. Launches include Piqray®, Adakveo® and Tabrecta® 2. Growth drivers include Promacta®/Revolade®, Tafinlar®+ Mekinist®, Kisqali®, Lutathera®, Kymriah® and Jakavi® (marketed by Novartis ex-US). 3. Base business – other brands. Gx include Afinitor®, Exjade® / Jadenu®, Glivec® and Sandostatin® All % growth relate to cc unless otherwise stated.

Key growth drivers accelerating in the US, while market still recovering from COVID-19

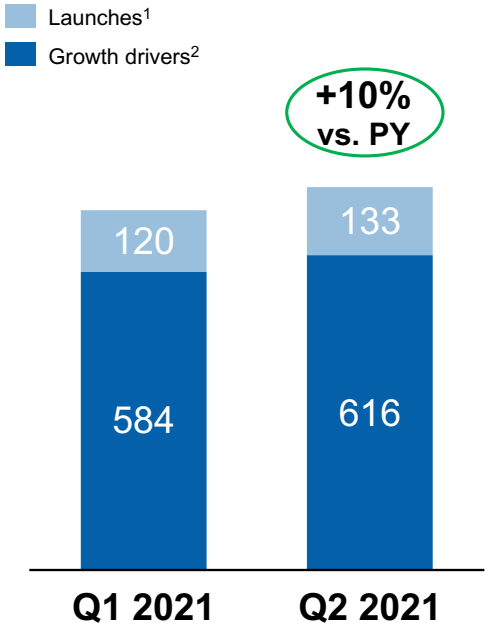
COVID-19 continues to weigh on US Oncology market

Biopsy rates remain suppressed (%)



Q2 showing partial recovery in US with growth drivers and recent launches accelerating

USD m, % cc



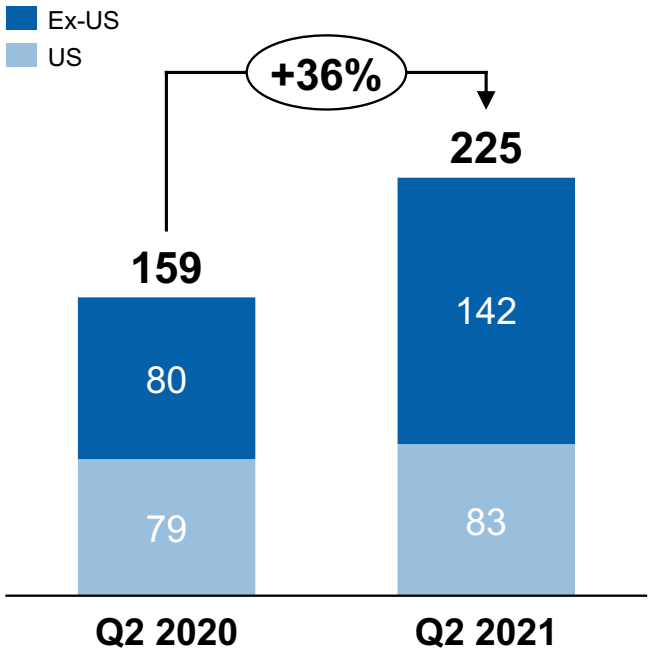
- Growth drivers and launches contribute 49% of sales (up from 47% Q1 2021)
- Promacta® USD 235m in Q2, +7% QoQ
- Kisqali® USD 83m in Q2, +17% QoQ
- Tafinlar®+Mekinist® USD 151m in Q2, +8% QoQ
- Piqray®, Tabrecta® and Adakveo® together USD 133m in Q2, +11% QoQ

1. Launches include Piqray®, Adakveo® and Tabrecta® 2. Growth drivers include Promacta®/Revolade®, Tafinlar®+ Mekinist®, Kisqali®, Lutathera®, Kymriah®

Kisqali® grew 36% in Q2 with share gains ex-US

Sales evolution

USD m, % cc



Strength of OS data in aBC is driving differentiation in the class

- Updated OS data from MONALEESA-3 presented at ASCO showed prolonged and consistent OS benefit with a median OS of ~4.5 years[1]
- Only CDK 4/6i to demonstrate OS benefit in 2 Ph3 trials and longest median OS

Kisqali® gaining growth momentum despite slow market recovery

- Ex-US: Continued strong double-digit growth, with uptake driven by patient share gains in Europe; market leader in pre-menopausal setting in France, Italy and Spain
- US: Q2 sales +17% vs. Q1, benefitting from higher demand due to increased field force reach coupled with targeted digital engagement

Potential to become the only CDK4/6i in intermediate and high-risk eBC

- NATALEE adjuvant study completed enrollment; readout expected in 2022

aBC – advanced breast cancer eBC – early breast cancer 1. Intent to treat population.

^{177}Lu-PSMA-617 launch preparations are progressing, ready to meet immediate launch demand upon approval in US

Prognosis remains poor for patients with mCRPC

2nd	Most diagnosed cancer in men[1]
>80%	Of patients metastatic at the time of CRPC diagnosis[2]
~10	Months median OS on available treatment options[3]

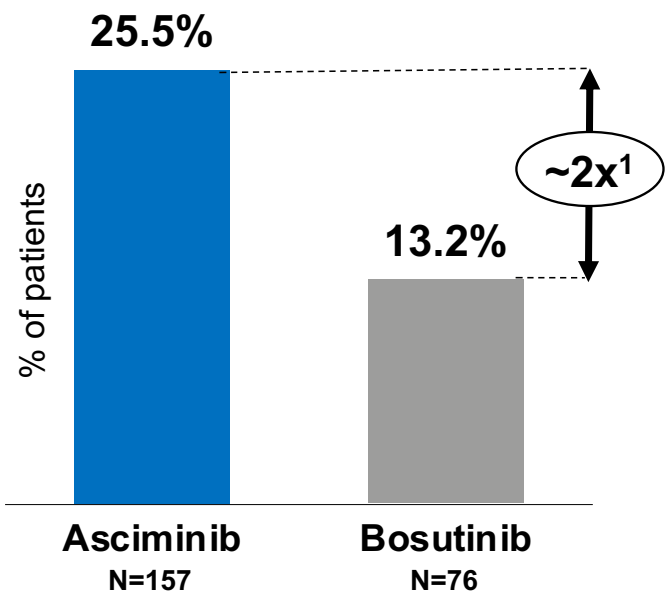
Building foundation for strong launch upon approval in US

- ^{177}Lu-PSMA-617 uniquely positioned to address unmet needs in mCRPC with strong data on OS (median OS 15.3 months) and rPFS
- Disease state education campaign on PSMA as an important phenotypic biomarker
- Growth in PSMA awareness from 31% to 66% over last 12 months[4]
- Focused on activating >200 top treatment centers at launch for PSMA
- Expecting gradual ramp-up due to diagnostic, process set-up, licensure
- FDA BTD granted in June 2021
- Filing to FDA on track for H2 2021

See slide 55 for references.

Preparing to launch asciminib, the first STAMP inhibitor with potential to transform the standard of care in CML

Asciminib Ph3 study in 3L+ CML: Nearly doubled MMR rate at Week 24



Unmet need in later lines of CML remains high

- ~15% of CML patients progress to 3L
- Up to 55% of patients are intolerant to a previous TKI

Ready to launch the first STAMP[2] inhibitor in H1 2022

- 75% aided awareness on asciminib / MoA ahead of launch
- Submissions to FDA and EMA in 3L CML achieved in June 2021
- 2 FDA BTDs, RTOR and Fast Track designation received
- Blockbuster potential in 3L CML (incl. T315I)

Potential to provide the best benefit-risk profile in 1L CML

- ~50% of patients relapse on imatinib or are refractory/intolerant to imatinib[3]
- >30% of patients suffer from TKI-related non-hematological AEs[4]
- In earlier lines of CML treatment, asciminib may prevent resistance to currently available TKIs by combatting emergence of mutations at BCR-ABL1 ATP binding site

Initiating Ph3 study of asciminib vs. investigator-selected TKI; FPFV in Q4 2021

See slide 55 for references.

For Oncology, 2021 is a breakthrough year of delivering transformative innovation

Readouts

✓ **^{177}Lu-PSMA-617**	Ph3 – mCRPC
✓ **Iptacopan**	Ph2 – PNH
✓ **Canakinumab**	Ph3 – NSCLC 2L
✓ **Alpelisib**	Ph3 – PROS
✓ KYMRIAH (tisagenlecleucel)	Ph2 – FL
✓ LUTATHERA	Ph3 – GEP NET (OS)
Canakinumab	Ph3 – NSCLC 1L
KYMRIAH (tisagenlecleucel)	Ph2 – aNHL 2L
KISQALI ribociclib	Ph3 – HR+ HER2- aBC (M-2 OS)

Submissions

✓ JAKAVI ruxolitinib	EU, JP in a/c GVHD
✓ TABRECTA (capmatinib) tablets	EU in NSCLC
✓ **Asciminib**	US, EU in CML 3L
^{177}Lu-PSMA-617	US, EU in mCRPC
KYMRIAH (tisagenlecleucel)	US, EU and JP in FL
KYMRIAH (tisagenlecleucel)	US in aNHL 2L
Canakinumab	US in NSCLC 1L[1]
Tislelizumab	US in 2L ESCC
Tislelizumab	US in NSCLC
Alpelisib	US in PROS

Designations

Asciminib	FDA BTD in 3L CML
Sabatolimab	FDA Fast Track in MDS
Alpelisib	EU Orphan designation in PROS
^{177}Lu-PSMA-617	FDA BTD in mCRPC

✓ Achieved ✓ Readout not supportive

1. Depending on timing of final read-out submission may move to early 2022

Harry Kirsch
Chief Financial Officer

Financial review and 2021 guidance

Solid H1 performance despite continued impact of COVID-19

Group[1] USD million	**Q2 2021**	**Change vs. PY**		**H1 2021**	**Change vs. PY**	
		% USD	**% cc**		**% USD**	**% cc**
Net Sales	12,956	14	9	25,367	7	3
Core Operating income	4,345	18	13	8,302	6	2
Operating income	3,479	48	41	5,894	16	12
Net Income	2,895	55	49	4,954	23	19
Core EPS (USD)	1.66	22	16	3.17	9	5
EPS (USD)	1.29	57	52	2.20	24	21
Free Cash Flow	4,235	17		5,832	3	

Q2 ex. PY forward purchasing de-stocking[2]: Sales +5%; Core OpInc +4%

1. Core results, constant currencies and free cash flow are non-IFRS measures. Further details regarding non-IFRS measures can be found starting on page 48 of the Condensed Financial Report. 2. Growth excluding prior year COVID-19 related forward purchasing reversal is a non-IFRS measure, an explanation for this measure can be found on page 61 of the Condensed Interim Financial Report. All % growth relate to cc unless otherwise stated.

Strong Q2 Innovative Medicines division performance

	Q2 2021				Q2 2021 ex. PY forward purchasing de-stocking[2]		
	Net sales change vs. PY % cc[1]	**Core OpInc** change vs. PY % cc[1]	**Core margin** %[1]	**Core margin** change vs. PY %pts cc[1]	**Net sales** change vs. PY %, cc[1]	**Core OpInc** change vs. PY %, cc[1]	**Core margin** change vs. PY %pts cc[1]
Innovative Medicines	10	14	37.3	1.3	7	6	-0.2
Sandoz	5	3	21.7	-0.4	-1	-8	-1.8
Group	9	13	33.5	1.2	5	4	-0.3

1. Core results, constant currencies and free cash flow are non-IFRS measures. Further details regarding non-IFRS measures can be found starting on page 48 of the Condensed Financial Report. 2. Growth ex. PY forward purchasing de-stocking is a non-IFRS measure; explanation can be found on page 61 of Condensed Interim Financial Report.

2021 Novartis full year guidance

Barring unforeseen events; growth vs. PY in cc

Group | full year guidance[1]

vs. PY (cc)

Group sales expected to grow **low to mid single digit**

- **IM Division** expected to **grow mid single digit**
- **Sandoz** expected to **decline low to mid single digit**

Group core operating income expected to grow **mid single digit, ahead of sales**

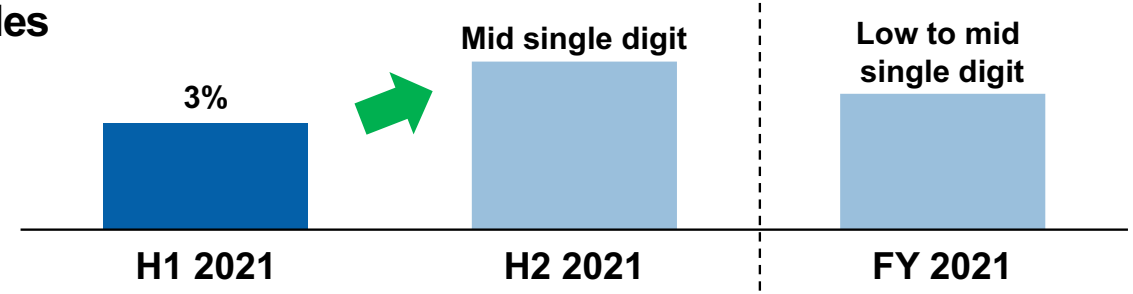
- **IM Division** expected to **grow mid to high single digit, ahead of sales**
- **Sandoz** expected to **decline low to mid teens**

1. Key assumptions:
   - Our guidance assumes that we see a continuation of the return to normal global healthcare systems including prescription dynamics, particularly oncology, in H2 2021
   - In addition, we assume that no Gilenya® and no Sandostatin® LAR generics enter in 2021 in the US

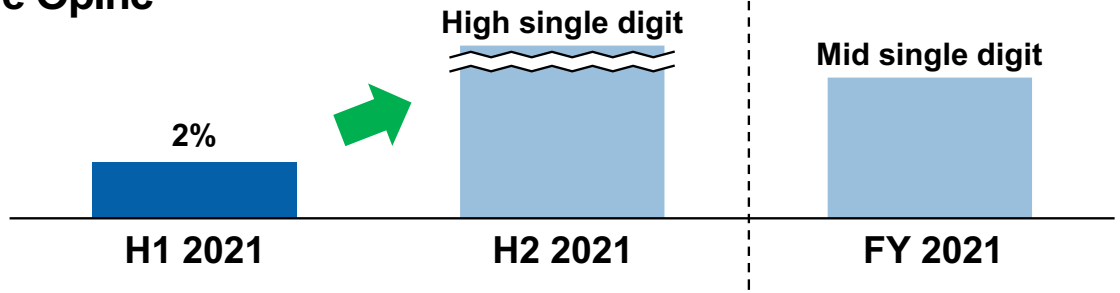
H2 2021 sales and core OpInc growth expected to accelerate as healthcare systems return to normal

Group growth vs. PY illustrative %pts, cc

Sales



Core OpInc



H2 2021 sales[1]

- Growth acceleration as we expect continuation of the return to normal global healthcare systems including prescription dynamics, particularly in oncology
- Sandoz stabilization

H2 2021 Core operating income

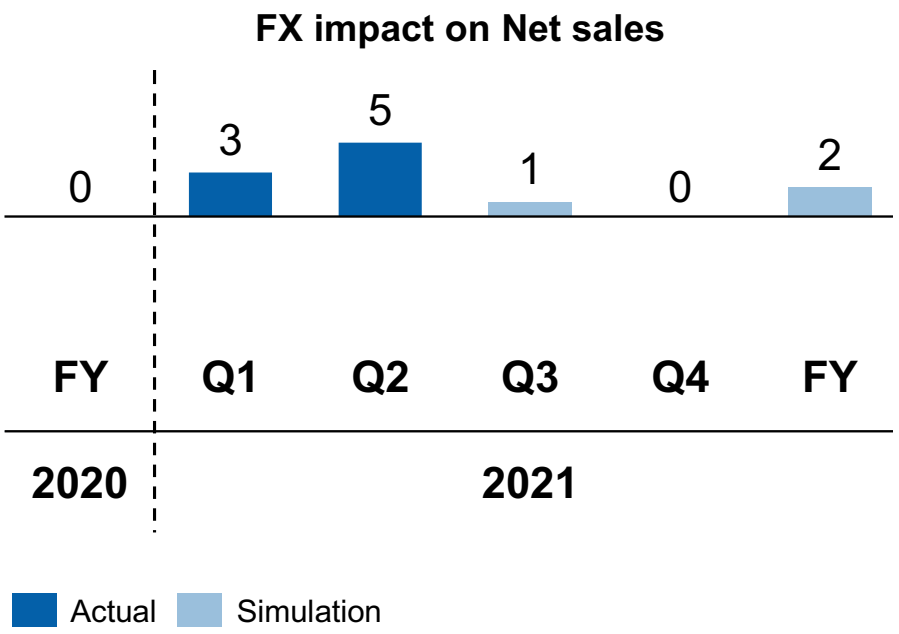
- Driven by higher sales
- Increased investments in growth drivers as markets reopen, and pipeline (incl. tislelizumab)
- Ongoing productivity programs

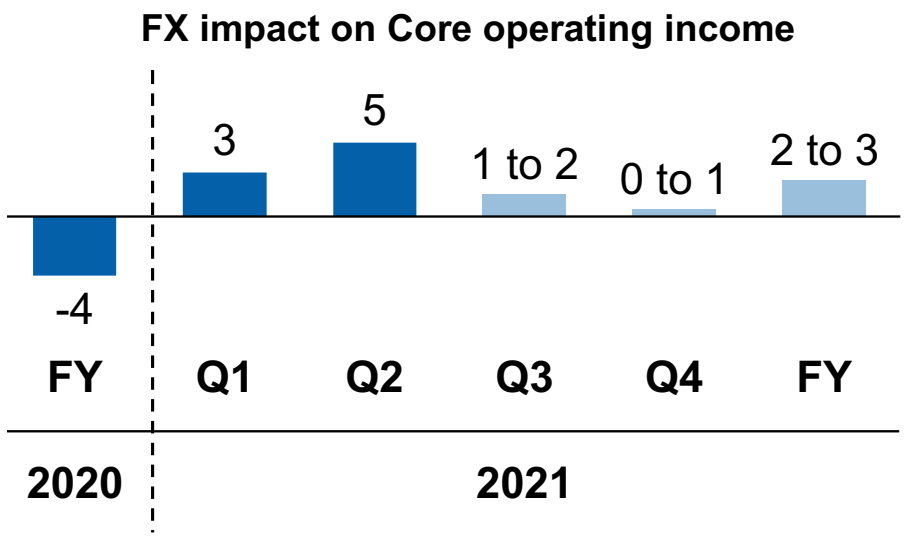
1. Assumes no Gilenya® and no Sandostatin® LAR generics enter in the US.

Expected currency impact for H2 and full year 2021

Currency impact vs. PY

%pts, assuming mid-July exchange rates prevail in 2021





Vas Narasimhan

Chief Executive Officer

2021 catalysts maintaining long-term momentum

✓ Achieved
✓ Readout not supportive

Potential catalysts	Selected examples			
Major approvals	**Kesimpta® (EU/JP)** ✓ RMS	**Entresto® (US)** ✓ HFpEF	**Cosentyx® (US ✓/JP/CN)** Pediatric psoriasis	
Major submissions[1]	**Alpelisib (BYL719)** PROS	**Asciminib (ABL001)** ✓ CML	**Jakavi®** ✓ Acute and chronic GvHD	**Beovu®** DME
	^{177}Lu-PSMA-617 mCRPC	**Kymriah®** FL	**Leqvio® (US)[2]** ✓ Hyperlipidemia	**Tislelizumab (VDT482)** 2L esophageal cancer, NSCLC
Major readouts Enabling submission 2021	**Kymriah®** aNHL 2L	**Canakinumab (ACZ885)[3]** NSCLC 1L	**Entresto®[4]** ✓ Post-AMI No submission planned	
Enabling submission 2022	**Ligelizumab (QGE031)[5]** CSU	**Cosentyx®** HS	**Sabatolimab (MBG453)[6]** MDS	
Others	**Iptacopan (LNP023)** ✓ Ph2 IgAN	**Iptacopan (LNP023)** ✓ Ph2 PNH	**Iptacopan (LNP023)** Ph2 C3G	**Kisqali®** Breast cancer (MONALEESA-2)
Pivotal study starts	**Iptacopan (LNP023)** ✓ Ph3 IgAN	**Iptacopan (LNP023)** ✓ Ph3 C3G	**Iptacopan (LNP023)** Ph3 aHUS	**Ligelizumab (QGE031)** Food allergy
	Ligelizumab (QGE031) CINDU	**^{177}Lu-PSMA-617** ✓ pre-taxane	**^{177}Lu-PSMA-617** ✓ mHSPC	

1. First submission in any market. 2. Resubmitted to FDA. 3. Depending on timing of final readout submission may move to early 2022. 4. Numerical trends consistently favored Entresto® vs. active comparator but did not meet primary composite endpoint. The safety profile of Entresto® was confirmed. No submission planned. 5. Q4/2021-Q1/2022 potential COVID impact. 6. Planned DMC readout for CR completed, study continues blinded to PFS readout, with submission in 2022/2023 using PFS and/or OS outcomes of Ph2 and/or Ph3 trial.

Consistent long-term performance driving confidence for the future

Strong performance in Q2 driven by the momentum of key growth drivers including Cosentyx®, Entresto®, Zolgensma® and Oncology portfolio

Pipeline of novel medicines continues to progress including positive readouts in diseases with high unmet need with iptacopan, Zolgensma® and ^{177}Lu-PSMA-617

Reconfirming FY 2021 guidance and our commitment to drive long-term accretive growth

Appendix

Key growth drivers and launches continue momentum in H1

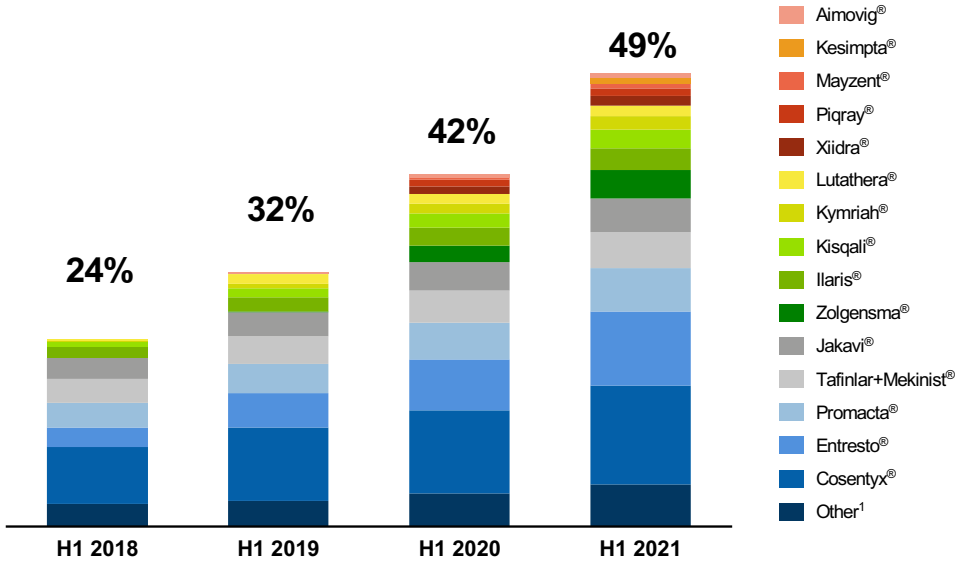
H1 key growth driver sales momentum[1]

	Sales USD Million	Growth vs. PY USD Million	Growth vs. PY cc
Entresto	1,675	526	40%
Cosentyx	2,228	354	16%
zolgensma	634	259	63%
PROMACTA (eltrombopag)	976	151	16%
JAKAVI ruxolitinib	761	133	14%
Kesimpta (ofatumumab)	116	116	nm
KISQALI ribociclib	420	100	28%
Xolair (omalizumab)	690	94	9%
ILARIS (canakinumab)	503	90	20%
KYMRIAH (tisagenlecleucel)	298	87	35%
Tafinlar + Mekinist	818	81	7%
MAYZENT (siponimod) tablets	124	60	89%

nm – not meaningful

Driving portfolio rejuvenation

Key growth drivers and launches
49% of IM sales, growing 24% in H1



1. Includes Xolair®, Beovu®, Adakveo®, Luxturna ®, Tabrecta® , Enerzair ®, Atectura® and Leqvio®

1. Innovative Medicines division. Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 48 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates in this Release refer to same period in prior year.

Solid H1 Innovative Medicines division performance

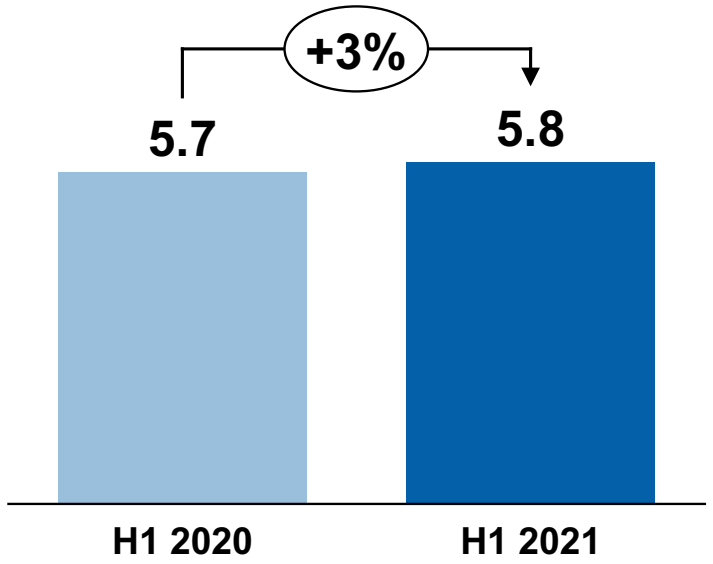
	H1 2021			
	Net sales change vs. PY % cc[1]	**Core OpInc** change vs. PY % cc[1]	**Core margin** %	**Core margin** change vs. PY %pts cc[1]
Innovative Medicines	5	6	36.8	0.4
Sandoz	-5	-19	20.5	-3.7
Group	3	2	32.7	-0.4

1. Core results, constant currencies and free cash flow are non-IFRS measures. Further details regarding non-IFRS measures can be found starting on page 48 of the Condensed Financial Report.

H1 2021 free cash flow growing to USD 5.8bn

Group free cash flow[1]

USD bn, % USD



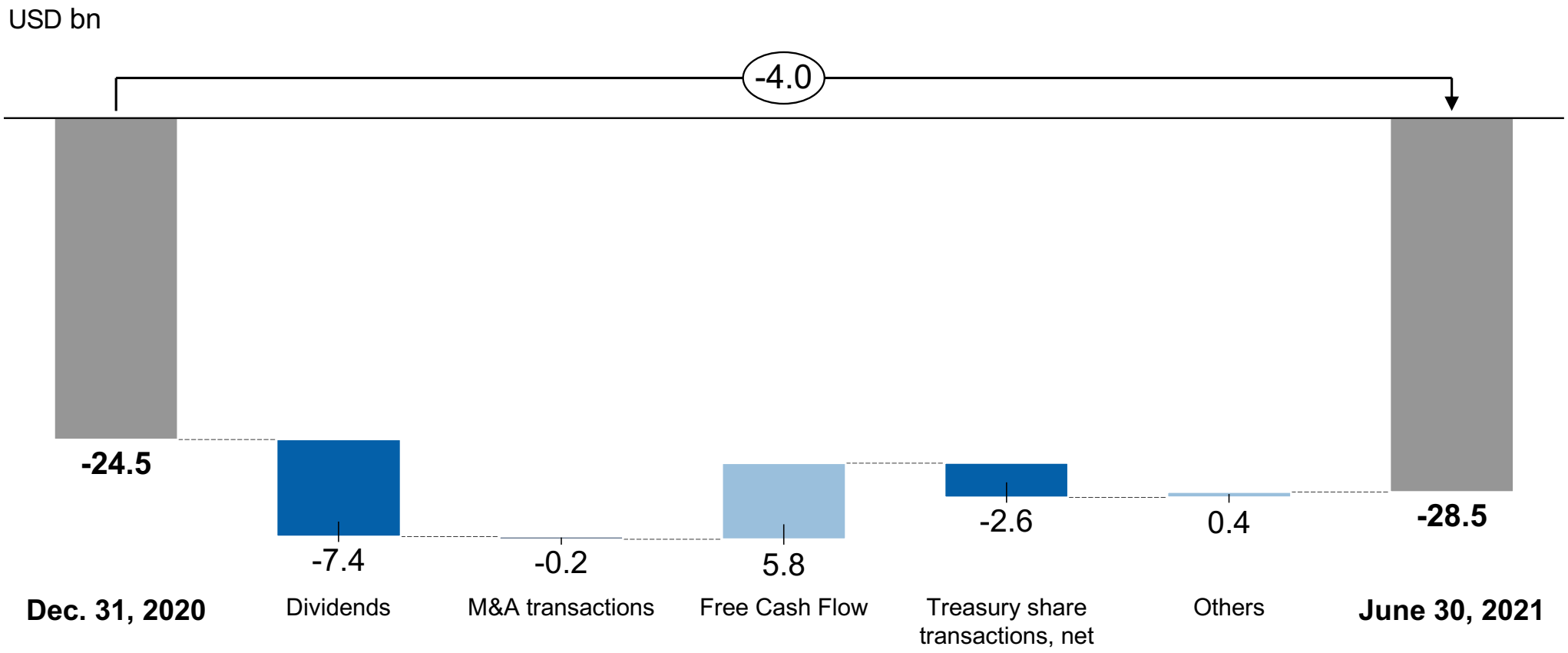
Key drivers vs. PY:

\+ Higher divestment proceeds

– Tislelizumab in-licensing (upfront payment USD 650m)

1. Free cash flow is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 48 of the Condensed Interim Financial Report.

Net debt increased to USD 28.5bn mainly due to dividend and buybacks, partly offset by strong FCF



2021 key pipeline milestones[1]

✓ **Achieved**
✓ **Readout not supportive**

	H1 2021			H2 2021		
Regulatory decisions and opinions	Entresto®	HFpEF (US)	✓	Cosentyx®	Pediatric psoriasis (US ✓/ CN / JP)	
	Kesimpta®	Relapsing MS (EU / JP)	✓			
Major expected submissions	Leqvio®	Hyperlipidemia (US)[2]	✓	Asciminib (ABL001)	CML 3L (JP)	
	Jakavi®	Acute and chronic GvHD (EU / JP)	✓	Beovu®	DME (JP)	
	Tabrecta®	NSCLC (EU)	✓	Alpelisib (BYL719)	PROS (US)	
	Beovu®	DME (US / EU)	H2-2021	Kymriah®	r/r Follicular lymphoma (US/EU/JP)	
	Asciminib (ABL001)	CML 3L (US /EU)	✓	^{177}Lu-PSMA-617	mCRPC (US/EU)	
	Cosentyx®	JIA (US /EU)	✓	Tislelizumab (VDT482)	2L esophageal cancer (US)	
				Tislelizumab (VDT482)	NSCLC (US)	
Major expected trial readouts*	Iptacopan (LNP023)	Ph2 - IgAN	✓	Canakinumab (ACZ885)	Ph3 - NSCLC 1L	
	Iptacopan (LNP023)	Ph2 - C3G	H2 2021[3]	ECF843	Ph2 - Dry eye	✓[8]
	Entresto®	Ph3 - Post-AMI	✓[4]	Ligelizumab (QGE031)	Ph3 - CSU[5]	
	Canakinumab (ACZ885)	Ph3 - NSCLC 2L	✓[6]	Kisqali®	Ph3 - aBC (MONALEESA-2 OS)	
	^{177}Lu-PSMA-617	Ph3 - mCRPC	✓	Remibrutinib (LOU064)	Ph2 - CSU	
	Cosentyx®	Ph3 - JIA	✓	Cosentyx®	Ph3 - HS	
				Sabatolimab (MBG453)	Ph2 - MDS[7]	
				Kymriah®	Ph3 - aNHL 2L	

*Achieved = on-time readout of data, irrespective of trial outcome. 1. 2021 Key milestone table may evolve based on read-out outcomes as well as BD&L activities. 2. Resubmitted to FDA 3. Ph2 interim data presented 4. Numerical trends consistently favored Entresto® vs. active comparator but did not meet primary composite endpoint. The safety profile of Entresto® was confirmed. No submission planned. 5. Q4/2021-Q1/2022 potential COVID impact. 6. Negative readout 7. Planned DMC readout for CR completed, study continues blinded to PFS readout, with submission in 2022/2023 using PFS and/or OS outcomes of Ph2 and/or Ph3 trial. 8. Program discontinued in broad population of moderate to severe DED.

Our pipeline projects at a glance

	Phase 1/2	Phase 3	Registration	Total
ONCOLOGY	44	26	3	73
PHARMACEUTICALS	58	23	3	84
Cardiovascular, Renal, Metabolism	6	7	1	14
Immunology, Hepatology, Dermatology	27	8	2	37
Neuroscience	6	2	0	8
Ophthalmology	5	3	0	8
Respiratory	7	2	0	9
Global Health	7	1	0	8
BIOSIMILARS	0	2	0	2
Total	**102**	**51**	**6**	**159**

Novartis pipeline in Phase 1 (1 of 2)

38 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)			
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Multiple solid tumors			
AAA602	^{177}Lu-PSMA-R2	Radioligand therapy target PSMA	Prostate cancer			
ADPT01	ADPT01	-	Colorectal cancer (combos)			
ADPT03	ADPT03	BCL11A	Sickle cell anemia			
CSJ137	CSJ137	Growth factor inhibitor	Anaemia			
DKY709	DKY709 + spartalizumab	Novel immunomodulatory agent	Cancers			
HDM201	HDM201 + MBG453, venetoclax	MDM2 inhibitor	Haematological malignancy			
JBH492	JBH492	-	Haematological malignancy			
JDQ443	JDQ443	KRAS Inhibitor	Solid tumors			
JEZ567	JEZ567	CD123 CAR-T	Acute myeloid leukaemia			
KAZ954	KAZ954	-	Solid tumors			
LXF821	LXF821	EGFR CAR-T	Glioblastoma multiforme			
LXH254	LXH254	cRAF inhibitor	Solid tumors (combo)			
MAK683	MAK683	EED inhibitor	Cancers			
MCM998	MCM998, LXG250	BCMA CAR-T, CD19 CAR-T	Multiple myeloma			
MIK665	MIK665	MCL1 inhibitor	Acute myeloid leukaemia (combo)			
NIS793	NIS793, spartalizumab	TGFB1 inhibitor	Solid tumors			
NIZ985	NIZ985, spartalizumab	IL-15 agonist	Solid tumors			
NZV930	NZV930, spartalizumab, NIR178	CD73 antagonist	Solid tumors			
PDR001	spartalizumab	PD1 inhibitor	Solid tumors (combo)			
PHE885	PHE885	BCMA cell therapy	Multiple Myeloma			
SQZ622	SQZ622	CD123xCD3 modulator	Acute myeloid leukaemia			
TNO155	TNO155	SHP2 inhibitor	Solid tumors (single agent)	Solid tumors (combo)	Solid tumors (combo)	
VAY736	ianalumab + ibrutinib	BAFF-R inhibitor	Haematological malignancy			
VOB560	VOB560	-	Cancers			
VPM087	gevokizumab	IL-1 beta antagonist	Colorectal cancer, 1st line			
WNT974	WNT974 + spartalizumab	Porcupine inhibitor	Solid tumors			
WVT078	WVT078	-	Multiple myeloma			
YTB323	YTB323 ± ibrutinib	CD19 CAR-T	Haematological malignancy			

Novartis pipeline in Phase 1 (2 of 2)

38 lead indications

Lead indication

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)
CEE321	CEE321	Pan JAK inhibitor	Atopic dermatitis
DFV890	DFV890	-	Anti-inflammatory therapy
FIA586	FIA586	-	Non-alcoholic steatohepatitis (NASH)
MHS552	MHS552	-	Autoimmune indications
MHV370	MHV370	-	Sjögren's, Systemic lupus erythematosus
NGI226	NGI226	-	Tendinopathy

Neuroscience

Code	Name	Mechanism	Indication(s)
OAV201	OAV201 (AVXS-201)	MECP2 gene therapy	Rett syndrome
NIO752	NIO752	Tau antagonist	Neurodegenerative diseases
LMI070	branaplam	mRNA splicing modulator	Huntington's disease

Ophthalmology

Code	Name	Mechanism	Indication(s)
MHU650	MHU650	-	Diabetic eye diseases

Respiratory Disease

Code	Name	Mechanism	Indication(s)
LTP001	LTP001	-	Respiratory diseases
NCJ424	NCJ424	-	Respiratory diseases

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)
MBL949	MBL949	-	Obesity related diseases

Global Health

Code	Name	Mechanism	Indication(s)
KAF156	ganaplacide	-	Malaria prophylaxis
INE963	INE963	-	Malaria, uncomplicated

Novartis pipeline in Phase 2

27 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)			
BYL719	alpelisib	PI3Kα inhibitor	PIK3CA-related overgrowth spectrum			
BLZ945	BLZ945	CSF-1R inhibitor	Solid tumors			
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	HGG/LGG, pediatrics			
INC280	Tabrecta®	Met inhibitor	Non-small cell lung cancer (Combo)			
INC424	Jakavi®	JAK1/2 inhibitor	Myelofibrosis (combination)		Acute GVHD, pediatrics	Chronic GVHD, pediatrics
LXH254	LXH254	cRAF inhibitor	Melanoma (combo)			
MBG453	sabatolimab	TIM3 antagonist	Unfit acute myeloid leukaemia			
NIR178	NIR178, spartalizumab	Ad2AR inhibitor, PD1 inhibitor	Cancers			
NIS793	NIS793	TGFB1 inhibitor	Pancreatic cancer			
PDR001	Spartalizumab	PD1 inhibitor	Metastatic melanoma (combo)			
SEG101	Adakveo®	P-selectin inhibitor	Sickle cell anaemia with crisis, pediatrics			

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)			
ADPT02	ADPT02	-	Non-alcoholic steatohepatitis (Combos)			
AIN457	Cosentyx®	IL17A inhibitor	Giant cell arteritis		Lichen planus	
CFZ533	iscalimab	CD40 inhibitor	Renal Tx	Sjögren's	Hidradenitis	Liver Tx
CMK389	CMK389	IL-18 inhibitor	Atopic dermatitis			
LJN452	tropifexor + licogliflozin	FXR agonist	Non-alcoholic steatohepatitis (Combos)			
LNA043	LNA043	ANGPTL3 agonist	Knee osteoarthritis			
LOU064	remibrutinib	BTK inhibitor	Chronic spontaneous urticaria		Sjögren's	
LRX712	LRX712	-	Osteoarthritis			
LYS006	LYS006	Anti-inflammatory	Acne	Colitis ulcerative	Hidradenitis	
MAS825	MAS825	-	NLRC4-GOF indications			
VAY736	ianalumab	BAFF-R inhibitor	Sjögren's	Autoimmune hepatitis		
			Systemic lupus erythematosus			

Neuroscience

Code	Name	Mechanism	Indication(s)			
BLZ945	BLZ945	CSF-1R inhibitor	Amyotrophic lateral sclerosis			
MIJ821	MIJ821	NR2B inhibitor	Depression			
OAV101	AVXS-101	Survival motor neuron (SMN) gene therapy	SMA IT[1)]			

1. Preclinical studies to address partial clinical hold completed.

Ophthalmology

Code	Name	Mechanism	Indication(s)		
CPK850	CPK850	RLBP1 AAV	Retinitis pigmentosa		
LKA651	LKA651	EPO inhibitor	Diabetic retinopathy		
SAF312	SAF312	TRPV1 antagonist	Chronic ocular surface pain		
UNR844	UNR844	Reduction of disulfide bonds	Presbyopia		

Respiratory Disease

Code	Name	Mechanism	Indication(s)			
CMK389	CMK389	IL-18 inhibitor	Pulmonary sarcoidosis			
CSJ117	CSJ117	TSLP inhibitor	Asthma			
QBW251	icenticaftor	CFTR potentiator	Chronic obstructive pulmonary disease			Bronchiectasis
QMF149	Atectura®	Combo	Asthma, pediatrics			

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)			
CFZ533	iscalimab	CD40 inhibitor	Lupus nephritis	Type 1 diabetes mellitus		
HSY244	HSY244	-	Atrial fibrillation			
LNP023	iptacopan	CFB inhibitor	Membranous nephropathy	Atypical haemolytic uraemic syndrome		

Global Health

Code	Name	Mechanism	Indication(s)			
AFQ056	mavoglurant	mGluR5 antagonist	Cocaine use disorder			
KAE609	cipargamin	PfATP4 inhibitor	Malaria, severe	Malaria, uncomplicated		
KAF156	ganaplacide	-	Malaria, uncomplicated			
LXE408	LXE408	Protozoan inhibitor	Visceral leishmaniasis			

Novartis pipeline in Phase 3

6 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)
AAA617	^{177}Lu-PSMA-617	Radioligand therapy target PSMA	Metastatic castration-resistant prostate cancer (mCRPC) mCRPC, pre-taxane Metastatic hormone sensitive prostate cancer (mHSPC)
AAA601[1]	Lutathera®	Radioligand therapy target SSTR	Gastroenteropancreatic neuroendocrine tumors, 1st line in G2/3 tumors (GEP-NET 1L G3)
ABL001	asciminib	BCR-ABL inhibitor	Chronic myeloid leukemia, 1st line
ACZ885	canakinumab	IL-1b inhibitor	Non-small cell lung cancer (NSCLC), 1L; NSCLC, adjuvant
BYL719	Piqray®	PI3Kα inhibitor	HER2+ adv BC; Triple negative breast cancer; Ovarian cancer
CTL019	Kymriah®	CD19 CAR-T	r/r Follicular lymphoma 1L high risk acute lymphocytic leukaemia, pediatrics & young adults Relapsed/refractory aggressive non-Hodgkin's lymphoma
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	Thyroid cancer
ETB115	Promacta®	Thrombopoietin receptor (TPO-R) agonist	Radiation sickness syndrome
LEE011	Kisqali®	CDK4/6 Inhibitor	HR+/HER2- BC (adj)
MBG453	Sabatolimab	TIM3 antagonist	Myelodysplastic syndrome
VDT482	tislelizumab	PD1 inhibitor	2L ESCC; Non-small cell lung cancer 1L Nasopharyngeal Carcinoma; 1L Gastric cancer 1L ESCC; Localized ESCC 1L Hepatocellular Carcinoma; 1L Small Cell Lung Cancer 1L Bladder Urothelial Cell Carcinoma

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Lupus Nephritis; AS H2H; Hidradenitis suppurativa Psoriatic arthritis (IV formulation) Ankylosing spondylitis (IV formulation)
QGE031	ligelizumab	IgE inhibitor	Chronic spontaneous urticaria; Food allergy Chronic inducible urticarial (CINDU)

Neuroscience

Code	Name	Mechanism	Indication(s)
AMG334	Aimovig®	CGRPR antagonist	Migraine, pediatrics
BAF312	Mayzent®	S1P1,5 receptor modulator	Multiple sclerosis, pediatrics

Respiratory Disease

Code	Name	Mechanism	Indication(s)
IGE025	Xolair®	IgE inhibitor	Food allergy; Auto-injector

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)
KJX839	Leqvio®	siRNA (regulation of LDL-C)	CVRR-LDLC; Hyperlipidemia, pediatrics
LCZ696	Entresto®	Angiotensin receptor/neprilysin inhibitor	Congestive heart failure, pediatrics[2]
LNP023	Iptacopan	CFB inhibitor	Paroxysmal nocturnal haemoglobinuria IgA nephropathy; C3 glomerulopathy
TQJ230	Pelacarsen	ASO targeting Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein (a) (CVRR-Lp(a))

Biosimilars

Code	Name	Mechanism	Indication(s)
GP2411	denosumab	anti RANKL mAb	Denosumab BioS
SOK583	aflibercept	VEGF inhibitor	Ophthalmology indication (as originator)

Ophthalmology

Code	Name	Mechanism	Indication(s)
RTH258	Beovu®	VEGF inhibitor	Diabetic retinopathy Retinal vein occlusion[3] Diabetic macular edema

Global Health

Code	Name	Mechanism	Indication(s)
COA566	Coartem®	-	Malaria, uncomplicated (<5kg patients)

1. ^{177}Lu-dotatate in US. 2. Approved in US. 3. Under evaluation.

Novartis pipeline in registration

2 lead indication

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)	
INC424	Jakavi®	JAK1/2 inhibitor	Acute GVHD	Chronic GVHD
ABL001	asciminib	BCR-ABL inhibitor	Chronic myeloid leukemia, 3rd line	

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Cosentyx 300mg auto-injector and pre-filled syringe Juvenile idiopathic arthritis

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)
KJX839	Leqvio®	siRNA (regulation of LDL-C)	Hyperlipidemia[1]

1. Approved in EU.

Novartis submission schedule

New Molecular Entities: Lead and supplementary indications

LEAD INDICATIONS

2021	2022	2023	2024	≥2025		
177Lu-PSMA-617 Lead AAA617 mCRPC 3L	**ligelizumab** Lead QGE031 CSU	**iptacopan** Lead LNP023 PNH	**SAF312** Lead COSP	**^{177}Lu-NeoB** Lead AAA603 Multiple Solid Tumors	**ganaplacide** Lead KAF156 Malaria uncomplicated	**MIJ821** Lead Depression
asciminib Lead ABL001 CML 3L	**sabatolimab**[1] Lead MBG453 HR-MDS		**UNR844** Lead Presbyopia	**^{177}Lu-PSMA-R2** Lead AAA602 Prostate cancer	**iscalimab** Lead CFZ533 Renal Tx	**NIS793** Lead Solid tumors
tislelizumab Lead VDT482 2L esophageal cancer				**branaplam** Lead LMI070 Huntington's disease	**ianalumab** Lead VAY736 Sjögren's syndrome	**OAV201** Lead AVXS-201 Rett syndrome
				CEE321 Lead Atopic Dermatitis	**icenticaftor** Lead QBW251 COPD	**pelacarsen** Lead TQJ230 CVRR-Lp(a)
				cipargamin Lead KAE609 Malaria severe	**LNA043** Lead Knee osteoarthritis	**remibrutinib** LOU064 CSU
				CPK850 Lead RP	**LXE408** Lead Visceral leishmaniasis	**spartalizumab** Lead PDR001 Metastatic melanoma (combo)
				CSJ117 Lead Asthma	**LXH254** Lead Solid tumors (combo)	**TNO155** Lead Solid tumors
				gevokizumab Lead VPM087 1st line CRC / 1st line RCC	**mavoglurant** Lead AFQ056 Cocaine use disorder	**tropifexor&licogliflozin** Lead LJN452 NASH (combos)

NEW INDICATIONS

2021	2022	2023		2024	≥2025	
tislelizumab LCM VDT482 NSCLC	**tislelizumab** LCM VDT482 1L Nasopharyngeal Carcinoma	**177Lu-PSMA-617** LCM AAA617 Pre-taxane	**tislelizumab** LCM VDT482 1L ESCC	**177Lu-PSMA-617** LCM AAA617 mHSPC	**asciminib** LCM ABL001 CML 1L	**iptacopan** LCM LNP023 iMN	**ligelizumab** LCM QGE031 Food allergy
		iptacopan LCM LNP023 C3G	**tislelizumab** LCM VDT482 Localized ESCC	**sabatolimab** LCM MBG453 Unfit AML	**cipargamin** LCM KAE609 Malaria uncomplicated	**iscalimab** LCM CFZ533 Liver Tx	**ligelizumab** LCM QGE031 CINDU
		iptacopan LCM LNP023 IgAN	**tislelizumab** LCM VDT482 1L Hepatocellular Carcinoma	**tislelizumab** LCM VDT482 1L Small Cell Lung Cancer	**ianalumab** LCM VAY736 AIH	**iscalimab** LCM CFZ533 Sjögren's syndrome	**remibrutinib** LCM LOU064 Sjögren's syndrome
		tislelizumab LCM VDT482 1L Gastric Cancer		**tislelizumab** LCM VDT482 1L Bladder Urothelial Cell Carcinoma	**iptacopan** LCM LNP023 aHUS		

1. Filing opportunity in 2022 / 2023, based on PFS and/or OS outcomes from a dual approach based on parallel Phase 2 and Phase 3 trials.

Novartis submission schedule

Supplementary indications for existing brands

2021	2022	2023	2024	≥2025		
alpelisib LCM BYL719 PROS	**Cosentyx** LCM secukinumab, AIN457 PsA IVIV	**canakinumab** LCM ACZ885 Adjuvant NSCLC	**Adakveo** LCM SEG101 Sickle cell anaemia with crisis ped	**Aimovig** LCM erenumab, AMG334 Pediatric Migraine	**Cosentyx** LCM secukinumab, AIN457 Lupus Nephritis	**Leqvio** LCM KJX839 CVRR-LDLC
Beovu LCM brolucizumab, RTH258 DME	**Cosentyx** LCM secukinumab, AIN457 AS H2H	**Cosentyx** LCM secukinumab, AIN457 AS IVIV	**Beovu** LCM brolucizumab, RTH258 RVO[4]	**aflibercept** BioS SOK583 Neovascular age-related macular degeneration	**Cosentyx** LCM secukinumab, AIN457 Lichen Planus	**Mayzent** LCM siponimod, BAF312 Pediatric MS
canakinumab[1] LCM ACZ885 NSCLC 1L	**Cosentyx** LCM secukinumab, AIN457 Hidradenitis suppurativa	**Denosumab** BioS GP2411 anti RANKL mAb	**Coartem** LCM artemether + lumefantrine, COA566 Malaria uncompl., formula for <5kg	**Beovu** LCM brolucizumab, RTH258 Diabetic retinopathy	**Jakavi** LCM ruxolitinib, INC424 Myelofibrosis (combination)	**Piqray** LCM alpelisib, BYL719 HER2+ adv BC
Cosentyx LCM secukinumab, AIN457 Juvenile idiopathic arthritis	**Entresto EU**[2] LCM sacubitril/valsartan, LCZ696 Pediatric CHF	**Kisqali** LCM ribociclib, LEE011 HR+/HER2- BC (adj)	**Cosentyx** LCM secukinumab, AIN457 GCA		**Kymriah** LCM tisagenlecleucel, CTL019 1L high risk ALL, pediatrics & young adults	**OAV101** LCM AVXS-101 SMA IT
Jakavi LCM ruxolitinib, INC424 Chronic GVHD	**Tafinlar + Mekinist** LCM dabrafenib + trametinib, DRB436 HGG/LGG - Pediatrics	**Lutathera** LCM [177]Lu-oxodotreotide[3] GEP-NET 1L G3	**Jakavi** LCM ruxolitinib, INC424 Pediatrics Acute GVHD			
Jakavi LCM ruxolitinib, INC424 Acute GVHD	**Xolair** LCM omalizumab, IGE025 Auto-injector	**Piqray** LCM alpelisib, BYL719 TNBC	**Jakavi** LCM ruxolitinib, INC424 Pediatrics Chronic GVHD			
Kymriah LCM tisagenlecleucel, CTL019 aNHL 2L		**Piqray** LCM alpelisib, BYL719 Ovarian cancer	**Leqvio** LCM KJX839 Ped Hyoerlipidemia			
Kymriah LCM tisagenlecleucel, CTL019 r/r Follicular lymphoma		**Promacta** LCM eltrombopag, ETB115 Radiation sickness syndrome	**Tafinlar + Mekinist** LCM dabrafenib + trametinib, DRB436 Thyroid cancer			
		Xolair LCM omalizumab, IGE025 Food allergy				

1. Depending on timing of final read-out submission may move to early 2022. 2. Approved in US. 3. 177Lu-dotatate in US. 4. Under evaluation.

References

Consistent long-term performance	
1	Cosentyx®, Entresto®, Zolgensma®, Kisqali®, Mayzent®, Tafinlar+Mekinist®, Jakavi®, Beovu®, Xiidra®, Aimovig®, Xolair®.
2	Lutathera®, Kymriah®, Piqray®, Adakveo®, Kesimpta®, Leqvio®, Tabrecta®, Asciminib.
3	Brands with 2024 consensus sales lower than 2019 actual sales (Glivec®, Tasigna®, Afinitor®, Votrient®, Promacta®, Exjade®, Sandostatin®, Galvus®, Gilenya®, Lucentis®).
Entresto®	
1	IQVIA National Prescription Audit
2	2021 Update to the 2017 ACC Expert Consensus Decision Pathway (ECDP) for Optimization of Heart Failure Treatment: Answers to 10 Pivotal Issues About Heart Failure With Reduced Ejection Fraction https://www.jacc.org/doi/10.1016/j.jacc.2020.11.022
3	Update to ESC heart Failure guidelines as presented at ESC-HF (28 Jun – 1 Jul 20201). Complete and final guideline to be released in August 2021
Leqvio®	
1	Data on file; American Heart Association. Center for Health Metrics and Evaluation. Accessed at: https://healthmetrics.heart.org/prevalence-and-number-of-us-adults-eligible-for-and-currently-using-statin-therapy-nhanes-2011-2014/; Wong ND. Journal of Clinical Lipidology. 2016;10(5):1109–1118; American Heart Association/American Stroke Association. Cardiovascular Disease: A Costly Burden
2	<70mg/dL
3	Non-statin lipid lowering therapies include ezetimibe and PCSK9i mAbs.
4	LAAD; IQVIA US Market Access Strategy Consulting.
5	Percentages in table refer to share of eligible US population
6	Medicaid, federal.
7	In patients with elevated LDL-C despite treatment with maximally tolerated statin therapy. V-INITIATE NCT04929249; V-INCEPTION NCT04873934
Asciminib	
1	Difference: 12.2% (95% confidence interval: 2.19, 22.30, two-sided p-value: 0.029) per the Cochran–Mantel–Haenszel test which is stratified by baseline major cytogenetic response status, cut-off date 25-May-2020
2	Specifically Targeting BCR-ABL Myristoyl Pocket.
3	Garcia-Gutierrez V and Hernandez-Boluda JC, *Front.Oncol.* 2019; 9:603
4	ELN recommendations 2019
^{177}Lu-PSMA-617	
1	Epidemiology of Prostate Cancer. Rawla P., World J Oncol. 2019;10(2):63-89
2	Characterizing the castration-resistant prostate cancer population: a systematic review. M. Kirby et all., Int J Clin Pract. 2011;65(11):1180–92
3	In men with progressive mCRPC after docetaxel and abiraterone and/or enzalutamide, Smith et al., Phase III Study of Cabozantinib in Previously Treated Metastatic Castration-Resistant Prostate Cancer: COMET-1, J Clin Oncol 34:3005-3013
4	Sartor et al, NEJM, 2021, DOI: 10.1056/NEJMoa210732
5	Novartis primary market research ATU, May 2020 & June 2021

Clinical Trials Update

Includes selected ongoing or recently concluded global trials of Novartis development programs/products which are in confirmatory development or marketed (typically Phase 2b or later).

For further information on all Novartis clinical trials, please visit: www.novartisclinicaltrials.com

Cardiovascular, Renal and Metabolic

Entresto® - Angiotensin II Receptor Neprilysin Inhibitor (ARNI)

Study	NCT02468232 PARALLEL-HF (CLCZ696B1301)	NCT02678312 PANORAMA HF (CLCZ696B2319)
Indication	Heart failure, reduced ejection fraction	Heart failure in pediatric patients
Phase	Phase 3	Phase 3
Patients	225	360
Primary Outcome Measures	Time to the first occurrence of the composite endpoint - either cardiovascular (CV) death or heart failure (HF) hospitalization	Part 1: Pharmacodynamics and pharmacokinetics of sacubitril/valsartan LCZ696 analytes Part 2: Efficacy and safety compared with enalapril
Arms Intervention	Sacubitril/valsartan 50 mg, 100 mg, 200 mg bid/placebo of enalapril Enalapril 2.5 mg, 5 mg, 10 mg bid / placebo of sacubitril/valsartan	Part 1: Sacubitril/valsartan 0.8 mg/kg or 3.1 mg/kg or both; 0.4 mg/kg or 1.6 mg/kg or both (single doses). Part 2: enalapril/placebo 0.2 mg/kg bid (ped. formulation 1mg/ml) and adult formulation (2.5, 5, 10 mg bid); Sacubitril/valsartan (LCZ696)/placebo: Ped. formulation granules (12.5, 31.25 mg in capsules); liquid formulation (1mg/ml and 4mg/ml concentration) and adult formulation (50, 100, 200 mg bid)
Target Patients	Japanese heart failure patients (NYHA Class II-IV) with reduced ejection fraction	Pediatric patients from 1 month to < 18 years of age with heart failure due to systemic left ventricle systolic dysfunction
Read-out Milesstone(s)	Primary: Q1-2019 (actual); Extension (open-label): Q1-2021 (actual)	2022; (Analysis of 110 pts from Part 2 formed the basis for pediatric submission in Apr-2019 and approval by the US FDA in Oct-2019 for the treatment of symptomatic HF with systemic left ventricular systolic dysfunction in children aged 1 year and older)
Publication	Q1-2022	TBD

Entresto® - Angiotensin II Receptor Neprilysin Inhibitor (ARNI)

Study	NCT02884206 PERSPECTIVE (CLCZ696B2320)	NCT02924727 PARADISE-MI (CLCZ696G2301)
Indication	Heart failure	Post-acute myocardial infarction
Phase	Phase 3	Phase 3
Patients	592	5670
Primary Outcome Measures	Change from baseline in the CogState Global Cognitive Composite Score (GCCS)	Time to the first occurrence of a confirmed composite endpoint (cardiovascular (CV) death, heart failure (HF) hospitalization, or outpatient heart failure)
Arms Intervention	Sacubitril/valsartan 50, 100, and 200 mg bid with placebo of valsartan Valsartan 40, 80, and 160 mg bid tablets with placebo for sacubitril/valsartan	Sacubitril/valsartan 50 mg, 100 mg, 200 mg bid; placebo for ramipril ; placebo for valsartan Ramipril 1.25 mg, 2.5 mg, and 5 mg bid; placebo for sacubitril/valsartan; placebo for valsartan
Target Patients	Patients with chronic heart failure with preserved ejection fraction	Post-AMI patients with evidence of LV systolic dysfunction and/or pulmonary congestion, with no known prior history of chronic HF
Read-out Milesstone(s)	2022	Q2-2021 (actual)
Publication	TBD	PARADISE-MI study design / baseline characteristics: published in Q2-2021 (actual) Data presentation at ACC Q2-2021 and plans for further scientific events

Entresto® - Angiotensin II Receptor Neprilysin Inhibitor (ARNI)

Study	NCT03066804 PARALLAX (CLCZ696D2302)	NCT03785405 (CLCZ696B2319E1 - extension study)
Indication	Heart failure, preserved ejection fraction	Heart failure in pediatric patients
Phase	Phase 3	Phase 3
Patients	2572	240
Primary Outcome Measures	Change in NT-proBNP from baseline to week 12 and change in 6 minute walk distance (6MWD) from baseline to Week 24	Number of participants with Adverse Events (AEs) and Serious Adverse Events (SAEs)
Arms Intervention	Sacubitril/valsartan 50 mg, 100 mg and 200 mg bid and matching placebo Enalapril 2.5 mg, 5 mg and 10 mg bid and matching placebo Valsartan 40 mg, 80 mg, 160 mg bid and matching placebo	Single arm, open label sacubitril/valsartan (pediatric formulation granules (12.5, 31.25 mg in capsules); liquid formulation (1mg/ml and 4mg/ml concentration) and adult formulation (50, 100, 200 mg bid))
Target Patients	Heart failure patients (NYHA Class II-IV) with preserved ejection fraction	Pediatric patients with heart failure due to systemic left ventricle systolic dysfunction who have completed study CLCZ696B2319
Read-out Milesstone(s)	2019 (actual)	2023
Publication	Primary data publication in High Tier Journal 2021	TBD

KJX839 - siRNA (regulation of LDL-C)

Study	NCT03060577 ORION-3 (CKJX839A12201E1)	NCT03705234 ORION-4 (CKJX839B12301)
Indication	Hypercholesterolemia inc. Atherosclerotic Cardiovascular Disease (ASCVD) and ASCVD risk equivalents Heterozygous Familial Hypercholesterolaemia (HeFH)	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH)
Phase	Phase 2	Phase 3
Patients	490	15000
Primary Outcome Measures	LDL-C reduction at Day 210 for Group 1 subjects Changes in other lipids and lipoproteins and reduction of LDL-C of more than 50% for patients that are above LDL-C goal ; longer term exposure and safety.	A composite of major adverse cardiovascular events, defined as: Coronary heart disease (CHD) death; Myocardial infarction; Fatal or non-fatal ischaemic stroke; or Urgent coronary revascularization procedure
Arms Intervention	Group 1 - inclisiran sodium 300mg sc on Day 1 and every 180 days thereafter for up to 4 years. Group 2- Evolocumab 140mg s.c. injection on Day 1 and every 2 weeks until Day 336, followed by inclisiran 300mg on Day 360, Day 450 and then every 6 months for a planned duration of 4 years.	Arm 1: every 6 month treatment KJX839 300mg (given by subcutaneous injection on the day of randomization, at 3 months and then every 6-months) for a planned median duration of about 5 years Arm 2: matching placebo (given bysubcutaneous injection on the day of randomization, at 3 months and then every 6-months) for a planned median duration of about 5 years.
Target Patients	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy	Patient population with mean baseline LDL-C ≥ 100mg/dL
Read-out Milesstone(s)	2022	2026
Publication	TBD	TBD

KJX839 - siRNA (regulation of LDL-C)

Study	NCT03814187 ORION-8 (CKJX839A12305B)	NCT03851705 ORION-5 (CKJX839A12302)
Indication	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH) and Homozygous Familial Hypercholesterolemia (HoFH)	Hypercholesterolemia inc. Homozygous Familial Hypercholesterolemia (HoFH)
Phase	Phase 3	Phase 3
Patients	2'991	56
Primary Outcome Measures	Proportion of subjects achieving prespecified low density lipoprotein cholesterol (LDL-C) targets at end of study Safety and tolerability profile of long term use of inclisiran	LDL-C reduction at Day 150 Changes in PCSK9, other lipids and lipoproteins
Arms Intervention	Inclisiran sodium 300mg on day 1 (placebo patients entered into study from ORION 9, 10 & 11) or placebo on Day 1 (inclisiran patients entered into study from ORION 9, 10 & 11) then inclisiran 300mg on Day 90 and every 6 months for a planned duation of 3 years	Part 1: inclisiran sodium 300mg on Day 1 and Day 90 or placebo on Day 1 and Day 90 Part 2: inclisiran on Day 180 for patients who were randomized to the placebo group only, inclisiran on Day 270 and then every 6 months for a planned duration of 2 years for all patients
Target Patients	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy and risk equivalents (patients from ORION 9, 10 & 11 studies)	Patients with HoFH with background statin +/- ezetimibe therapy
Read-out Milesstone(s)	2023	Primary: Q3-2020 (actual); Final: H2-2021
Publication	TBD	TBD

KJX839 - siRNA (regulation of LDL-C)

Study	NCT04652726 ORION-16 (CKJX839C12301)	NCT04659863 ORION-13 (CKJX839C12302)
Indication	Hyperlipidemia, pediatrics	Hyperlipidemia, pediatrics
Phase	Phase 3	Phase 3
Patients	150	15
Primary Outcome Measures	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to Day 330	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to day 330
Arms Intervention	Group 1: Inclisiran sodium 300mg on Days 1, 90, 270, placebo on Day 360, inclisiran sodium 300mg on Days 450 and 630; Group 2: Placebo on Days 1, 90, 270, inclisiran sodium 300mg on Days 360, 450 and 630.	Group 1: Inclisiran sodium 300mg on Days 1, 90, 270, placebo on Day 360, inclisiran sodium 300mg on Days 450 and 630; Group 2: Placebo on Days 1, 90, 270, inclisiran sodium 300mg on Days 360, 450 and 630.
Target Patients	Adolescents (12 to less than 18 years) with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C)	Adolescents (12 to less than 18 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDL-C)
Read-out Milesstone(s)	2023	2023
Publication	TBD	TBD

LNP023 - Factor B inhibition of the complement alternative pathway

Study	NCT03373461 (CLNP023X2203)	NCT03439839 (CLNP023X2201)
Indication	IgA nephropathy (IgAN)	Paroxysmal nocturnal hemoglobinuria (PNH)
Phase	Phase 2	Phase 2
Patients	112	16
Primary Outcome Measures	Change from baseline of log transformed UPCR derived from the 24h urine collections at Baseline and Day 90	Reduction of chronic hemolysis, based on LDH level at Week 13
Arms Intervention	Placebo LNP023 Dose 1 LNP023 Dose 2 LNP023 Dose 3 LNP023 Dose 4	10 patients receiving LNP023 high dose daily over up to approximately 3 years 5 patients receiving LNP023 low dose daily over up to approximately 3 years
Target Patients	Patients with biopsy-verified IgA nephropathy	Patients with PNH, showing signs of active hemolysis despite treatment with SoC (defined as an antibody with anti C5 activity).
Read-out Milesstone(s)	H1-2021 (actual)	Primary: Q2-2020 (actual) Extension: 2023
Publication	Barratt et al. 2021. Oral Presentation at the 58th ERA-EDTA congress (Late Breaking Clinical Trials), June 6: IA2 results.	Antonio M. Risitano, MD, PhD1 et al. Presented at EBMT 2020 congress Jan 2021Pubs: Lancet Haematol - Study of Safety, Efficacy, Tolerability, Pharmacokinetics and Pharmacodynamics of LNP023 in in Patients With Paroxysmal Nocturnal Hemoglobinuria (PNH)

LNP023 - Factor B inhibition of the complement alternative pathway

Study	NCT03832114 (CLNP023X2202)	NCT03896152 (CLNP023X2204)
Indication	C3 glomerulopathy (C3G)	Paroxysmal nocturnal hemoglobinuria (PNH)
Phase	Phase 2	Phase 2
Patients	27	13
Primary Outcome Measures	Cohort A: Ratio to Baseline of UPCR to Week 12 derived from 24hr urine collection Cohort B: Change from Baseline in C3 Deposit Score (based on immunofluorescence microscopy) at Week 12	Reduction of PNH associated hemolysis, based on percentage of patients with 60% reduction in LDH or LDH below upper limit of normal up to 12 weeks of treatment.
Arms Intervention	Increasing doses of LNP023 up to 200mg bid: Cohort A: Native kidney patients Cohort B: Kidney transplanted patients	approximately 2 year Treatment with low LNP023 dose approximately 2 year Treatment with higher LNP023 dose
Target Patients	Patients with C3 glomerulopathy	Patients with PNH, showing signs of active hemolysis, not treated with any other complement inhibitor less than 3 months prior to study start Day 1
Read-out Milesstone(s)	H1-2021 (interim actual)	Primary: Q2-2020 (actual) Extension: 2022
Publication	Actual: Interim analysis data from Cohort-A presented at American Society of Nephrology (ASN 2020). Planned: Note not to be communicated externally until accepted. 1) Planned abstract at ERA-EDTA, Q3 2021 2) Planned abstract at ASN, Q4 2021	-Jang JH, et al. Presented at Korean Society of Hematology International Conference and 62nd Annual Meeting (ICKSH 2021) -Presented as an oral presentation (encore) at the European Haematology Association (EHA 2021) congress -Planned manuscript submission in Q3 2021

LNP023 - Factor B inhibition of the complement alternative pathway

Study	NCT03955445 (CLNP023B12001B)	NCT04154787 (CLNP023D12201)
Indication	C3 glomerulopathy (C3G)	Idiopathic membranous nephropathy (iMN)
Phase	Phase 2	Phase 2
Patients	27	72
Primary Outcome Measures	Characterize the effect of LNP023 treatment on a composite renal response endpoint at 9 months (1. a stable or improved eGFR and, 2. a reduction in proteinuria and 3. an increase in C3 compared to the CLNP023X2202 baseline visit)	Change from baseline of UPCR derived from 24hr urine collections at Baseline and Week 24
Arms Intervention	Open-label LNP023 200mg bid	LNP023 low dose LNP023 high dose Rituximab
Target Patients	Patients with C3 glomerulopathy	Patients with biopsy proven iMN who are at high risk of disease progression defined on the basis of antibody anti-PLA2R titre and proteinuria
Read-out Milesstone(s)	2025	2023
Publication	Wong et al 2021 Nephrology, Dialysis and Transplantation Vol. 36, Suppl. 1: eGFR trajectory	TBD

LNP023 - Factor B inhibition of the complement alternative pathway

Study	NCT04558918 APPLY-PNH (CLNP023C12302)	NCT04578834 APPLAUSE-IgAN (CLNP023A2301)
Indication	Paroxysmal nocturnal haemoglobinuria	IgA nephropathy
Phase	Phase 3	Phase 3
Patients	91	450
Primary Outcome Measures	Percentage of participants achieving a sustained increase in hemoglobin levels of ≥ 2 g/dL in the absence of red blood cell transfusions Percentage of participants achieving sustained hemoglobin levels ≥12 g/dL in the absence of red blood cell transfusions	Ratio to baseline in urine protein to creatinine ratio (sampled from 24h urine collection) at 9 months Annualized total estimated Glomerular Filtration Rate (eGFR) slope estimated over 24 months
Arms Intervention	Arm 1: Drug: LNP023, taken orally b.i.d. dosage supplied: 200 mg dosage form: hard gelatin capsule Route of Administration: Oral Arm 2: Drug: Eculizumab, administered as intravenous infusion every 2 weeks as per the stable regimen, the maintenance dose is a fixed dose. Dosage supplied: 300 mg/30mL Dosage form: Concentrate solution for infusion Drug: Ravulizumab, administered as intravenous infusion every 8 weeks, the maintenance dose is based on body weight. Dosage Supplied: 300 mg/30mL Dosage form: Concentrate solution for infusion	Arm 1 - LNP023 200mg BID Arm 2 - Placebo BID
Target Patients	Adult patients with PNH and residual anemia, despite treatment with an intravenous Anti-C5 antibody	Primary IgA Nephropathy patients
Read-out Milesstone(s)	Primary 2022	2023
Publication	TBD	Perkovic et al. 2021, Nephrology Dialysis Transplantation, Vol. 36, Suppl. 1: Study Design Wong et al. 2021, Nephrology Dialysis Transplantation, Vol. 36, Suppl. 1: IPTACOPAN (LNP023): A NOVEL ORAL COMPLEMENT ALTERNATIVE PATHWAY FACTOR B INHIBITOR SAFELY AND EFFECTIVELY STABILISES EGFR IN C3 GLOMERULOPATHY

LNP023 - Factor B inhibition of the complement alternative pathway

Study	NCT04817618 APPEAR-C3G (CLNP023B12301)	NCT04820530 APPOINT-PNH (CLNP023C12301)
Indication	C3 glomerulopathy	Paroxysmal nocturnal haemoglobinuria
Phase	Phase 3	Phase 3
Patients	68	40
Primary Outcome Measures	Log-transformed ratio to baseline in UPCR (sampled from a 24 hour urine collection)	Proportion of participants achieving a sustained increase from baseline in hemoglobin levels of ≥ 2 g/dL assessed , in the absence of red blood cell transfusions
Arms Intervention	Experimental: iptacopan 200mg b.i.d. Placebo Comparator: Placebo to iptacopan 200mg b.i.d.	Iptacopan (LNP023), taken orally b.i.d. (dosage supplied: 200mg)
Target Patients	Patients with native C3G	PNH patients who are naive to complement inhibitor therapy, including anti-C5 antibody
Read-out Milesstone(s)	2023	2023
Publication	TBD	TBD

TQJ230 - Antisense oligonucleotide targeting apolipoprotein(a) mRNA

Study	NCT04023552 Lp(a)HORIZON (CTQJ230A12301)
Indication	Cardiovascular risk reduction
Phase	Phase 3
Patients	7680
Primary Outcome Measures	Time to the first occurrence of MACE (cardiovascular death, non-fatal MI, non-fatal stroke and urgent coronary re-vascularization)
Arms Intervention	TQJ230 80 mg injected monthly subcutaneously or matched placebo
Target Patients	Patients with a history of Myocardial infarction or Ischemic Stroke, or a clinically significant symptomatic Peripheral Artery Disease, and Lp(a) ≥ 70 mg/dL
Read-out Milesstone(s)	2024
Publication	TBD

Immunology, Hepatology & Dermatology

CFZ533 - Blocking, non-depleting, Fc-silent, anti-CD40 monoclonal antibody

Study	NCT03781414 CONTRAIL I (CCFZ533A2202)
Indication	Liver transplantation
Phase	Phase 2
Patients	128
Primary Outcome Measures	Proportion of patients with composite event (BPAR, Graft Loss or Death) over 12 months
Arms Intervention	Control/Standard of Care: TAC + MMF + Corticosteroids CFZ533 dose A + MMF + Corticosteroids CFZ533 dose B + MMF + Corticosteroids
Target Patients	Liver transplant recipients
Read-out Milesstone(s)	2023
Publication	2023

CFZ533 - Blocking, non-depleting, Fc-silent, anti-CD40 monoclonal antibody

Study	NCT03663335 CIRRUS I (CCFZ533A2201)	NCT03905525 TWINSS (CCFZ533B2201)
Indication	Kidney transplantation	Sjögren's syndrome
Phase	Phase 2	Phase 2
Patients	325	260
Primary Outcome Measures	Proportion of patients with composite event (BPAR, Graft Loss or Death) at M12	Change in EULAR Sjögren's syndrome Disease Activity Index (ESSDAI) score and EULAR Sjögren's syndrome Patient Reported Index (ESSPRI) score
Arms Intervention	Two cohorts: de novo TX and maintenance Test Arms: CFZ533 + MMF + corticosteroids Standard of Care: TAC + MMF + corticosteroids	Three dose arms of CFZ533 Placebo
Target Patients	Kidney transplant recipients	Patients with Sjögren's syndrome
Read-out Milesstone(s)	2022	2022
Publication	2022	2022

Cosentyx® - Anti IL-17

Study	NCT03031782 (CAIN457F2304)
Indication	Psoriatic arthritis
Phase	Phase 3
Patients	80
Primary Outcome Measures	Time to 33 flares
Arms Intervention	Secukinumab (pre-filled syringe) 75 mg Placebo
Target Patients	Juvenile idiopathic arthritis subtypes of psoriatic and enthesitis-related arthritis
Read-out Milesstone(s)	H1-2021
Publication	H2-2021

Cosentyx® - Anti IL-17

Study	NCT03259074 SURPASS (CAIN457K2340)	NCT03504852 (CAIN457A2324)
Indication	Ankylosing spondylitis	Psoriasis
Phase	Phase 3	Phase 3B
Patients	837	331
Primary Outcome Measures	No radiographic structural progression as measured by modified Stoke Ankylosing Spondylitis Spine Score (mSASSS)	PASI 90 response and IGA mod 2011 0 or 1 response after 16 weeks of treatment
Arms Intervention	Secukinumab 150/300 mg Adalimumab biosimilar 40 mg	Secukinumab 300 mg every 2 weeks after weekly doses till Week 4 Secukinumab 300 mg every 4 weeks after weekly doses till Week 4
Target Patients	Patients with active ankylosing spondylitis	Subjects (≥90kg) with moderate to severe plaque psoriasis
Read-out Milesstone(s)	2022	Q3-2020 (actual)
Publication	Study design manuscript published. Baraliakos et al. Clinical Drug Investigation (2020) 40:269-278.	Publication (primary efficacy) in Q3-2021 (ongoing) Publication of 52-week planned in Q4-2021 (planned) Abstract at AAD in Q2-2021 Reich K. et al Presented at 2021 AAD VMX (LBA). Selected to be highlighted in the AAD "Meeting News",

Cosentyx® - Anti IL-17

Study	NCT03589885 MATURE (CAIN457A2325)	NCT03668613 (CAIN457A2311)
Indication	Psoriasis	Psoriasis
Phase	Phase 3	Phase 3
Patients	122	84
Primary Outcome Measures	PASI 75 response and IGA mod 2011 0 or 1 response after 12 weeks of treatment	Psoriasis Area and Severity Index (PASI) 75 response and Investigators' Global Assessment (IGA) 0 or 1 response at week 12
Arms Intervention	Secukinumab 2 mL (300 mg) auto-injector Secukinumab 2 x 1 mL (150 mg each) prefilled syringe Placebo 2 mL auto-injector Placebo 2 x 1 mL prefilled syringe	Secukinumab low dose Secukinumab high dose
Target Patients	Subjects with moderate to severe plaque psoriasis	Pediatric patients of age 6 to <18 years, with moderate to severe plaque psoriasis
Read-out Milesstone(s)	Final: Q4-2020	2023
Publication	Sigurgeirsson et al, Presentation at AAD VMX 2021 (16-week) IFPA 2021 (planned) EADV 2021 (planned) 52-week publication H2-2021	24-week paper publication in Q3 2021 (estimate). Magnolo et al. Presentation at AAD VMX 2021. Magnolo et al. Presentation at ESPD 2021. SPD (Q3 2021 - planned) EADV (Q3 2021 - planned) - pooled A2310/A2311 PG2C (Q4 2021 - planned) - pooled A2310/A2311

Cosentyx® - Anti IL-17

Study	NCT03713619 SUNSHINE (CAIN457M2301)	NCT03713632 SUNRISE (CAIN457M2302)
Indication	Hidradenitis Suppurativa (HS)	Hidradenitis Suppurativa (HS)
Phase	Phase 3	Phase 3
Patients	471	471
Primary Outcome Measures	Proportion of participants with Hidradenitis Suppurativa clinical response (HiSCR)	Proportion of patients with Hidradenitis Suppurativa Clinical Response (HiSCR)
Arms Intervention	Secukinumab 300 mg every 2 weeks Secukinumab 300 mg every 4 weeks Placebo (every 2 weeks) Placebo (every 4 weeks)	Secukinumab 300 mg every 2 weeks Secukinumab 300 mg every 4 weeks Placebo (every 2 weeks) Placebo (every 4 weeks)
Target Patients	Patients with moderate to severe Hidradenitis Suppurativa	Subjects with moderate to severe Hidradenitis Suppurativa
Read-out Milesstone(s)	Primary (week 16): H2-2021; Final: 2022	Primary (week 16): H2-2021; Final: 2022
Publication	Study design SHSA 2020; Primary 2022	Study design SHSA 2020; Primary 2022

Cosentyx® - Anti IL-17

Study	NCT03769168 (CAIN457F2304E1 - extension study)	NCT04156620 INVIGORATE-1 (CAIN457P12301)
Indication	Psoriatic arthritis	Axial spondyloarthritis
Phase	Phase 3	Phase 3
Patients	64	500
Primary Outcome Measures	Number of participants with JIA ACR30 response	The proportion of subjects achieving an ASAS40 (Assessment of SpondyloArthritis International Society criteria) response
Arms Intervention	Secukinumab 75 mg/0.5 ml Secukinumab 150 mg/1.0 ml	Secukinumab intravenous (i.v.) regimen Placebo intravenous (i.v.) regimen
Target Patients	Patients with juvenile idiopathic arthritis subtypes of juvenile psoriatic arthritis and enthesitis related arthritis	Patients with active axial spondyloarthritis
Read-out Milesstone(s)	2025	Primary (week 16): 2022; Final: 2023
Publication	TBD	2023

Cosentyx® - Anti IL-17

Study	NCT04179175 (CAIN457M2301E1)	NCT04181762 SELUNE (CAIN457Q12301)
Indication	Hidradenitis Suppurativa (HS)	Lupus Nephritis
Phase	Phase 3	Phase 3
Patients	745	460
Primary Outcome Measures	Proportion of patients with Hidradenitis Suppurativa Clinical Response (HiSCR)	Proportion of subjects achieving protocol-defined CRR
Arms Intervention	Secukinumab 300 mg every 2 weeks Secukinumab 300 mg every 4 weeks	Secukinumab 300 mg s.c. Placebo s.c.
Target Patients	Patients with moderate to severe hidradenitis suppurativa completing either of the core trials AIN457M2301 (NCT 0313632) or AIN567M2302 (NCT03713619)	Patients with active lupus nephritis (ISN/RPS Class III or IV, with or without co-existing class V features)
Read-out Milesstone(s)	2025	2026
Publication	Study design SHSA 2020	2026

Cosentyx® - Anti IL-17

Study	NCT04209205 INVIGORATE-2 (CAIN457P12302)	NCT04300296 PRELUDE (CAIN457S12201)
Indication	Psoriatic Arthritis (PsA)	Lichen Planus
Phase	Phase 3	Phase 2
Patients	380	108
Primary Outcome Measures	The proportion of subjects achieving American College of Rheumatology 50 (ACR50) response criteria	Proportion of patients achieving Investigator's Global Assessment (IGA 0/1) score at 16 weeks +30% delta vs placebo
Arms Intervention	Secukinumab intravenous (i.v.) regimen Placebo intravenous (i.v.) regimen	Secukinumab 300 mg s.c. Placebo s.c.
Target Patients	Patients with active psoriatic arthritis (PsA) despite current or previous NSAID, DMARD and/or anti-TNF therapy	Adult patients with biopsy-proven lichen planus not adequately controlled by topical therapies
Read-out Milesstone(s)	2022	2022
Publication	2023	TBD

LJN452 - FXR Agonist

Study	NCT04065841 ELIVATE (CLJN452D12201C)
Indication	Non-alcoholic steatohepatitis (NASH)
Phase	Phase 2
Patients	380
Primary Outcome Measures	Proportion of patients with resolution of NASH and no worsening of fibrosis OR improvement in fibrosis by at least one stage without worsening of NASH at Week 48 compared with baseline
Arms Intervention	Arm A: combination therapytropifexor + licogliflozin Arm B: tropifexor monotherapytropifexor + licogliflozin placebo Arm C: licogliflozin monotherapylicogliflozin + tropifexor placebo Arm D: licogliflozin placebo + tropifexor placebo
Target Patients	Adult patients with biopsy based non-alcoholic steatohepatitis (NASH) and liver fibrosis
Read-out Milesstone(s)	2023
Publication	2023

LNA043 - ANGPTL3 Agonist

Study	NCT03275064 (CLNA043X2202)	NCT04864392 ONWARDS (CLNA043A12202)
Indication	Knee osteoarthritis	Knee osteoarthritis
Phase	Phase 2	Phase 2
Patients	133	550
Primary Outcome Measures	Articular cartilage bi-layer collagen organisation evaluated with MRI and measured in milliseconds (ms) (Part A only) Number of patients with any adverse events, serious adverse events and death (Part A and Part B) Change in cartilage volume/thickness in the index region (Part B only)	Change from baseline in the cartilage thickness of the medial compartment of the knee as assessed by imaging
Arms Intervention	LNA043 40 mg Part B LNA043 20 mg Part B LNA043 20 mg Part A Placebo Part A Placebo Part B	LNA043 injection to the knee with dosing regimen A LNA043 injection to the knee with dosing regimen B LNA043 injection to the knee with dosing regimen C LNA043 injection to the knee with dosing regimen D Placebo injection to the knee
Target Patients	Patients with cartilage lesions of the knee (Part A) and knee osteoarthritis (Part B)	Patients with Symptomatic knee osteoarthritis
Read-out Milesstone(s)	2022	Primary 2024
Publication	TBD	TBD

LOU064 - Bruton's tyrosine kinase (BTK) inhibitor

Study	NCT03926611 (CLOU064A2201)	NCT04109313 (CLOU064A2201E1)
Indication	Chronic spontaneous urticaria (CSU)	Chronic spontaneous urticaria (CSU)
Phase	Phase 2	Phase 2
Patients	308	250
Primary Outcome Measures	Change from baseline in weekly Urticaria Activity Score (UAS7) at Week 4	Long-term safety and tolerability
Arms Intervention	Arm 1 Low dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 Arm 2 Medium dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 Arm 3 High dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 Arm 4 Low dose of LOU064 orally, twice daily from Day 1 to 85 Arm 5 Medium dose of LOU064 orally, twice daily from Day 1 to 85 Arm 6 High dose of LOU064 orally, twice daily from Day 1 to 85 Placebo arm Matching placebo, orally, twice daily from Day 1 to 85	Selected dose of LOU064 taken orally twice a day (morning and evening) from day 1 to week 52
Target Patients	Adults with CSU inadequately controlled by H1-antihistamines	Patients with CSU who have participated in preceding studies with LOU064
Read-out Milesstone(s)	H2-2021	2022
Publication	H2-2021	TBD

QGE031 - Anti-IgE

Study	NCT04210843 (CQGE031C2302E1)	NCT02649218 (CQGE031C2201E1)
Indication	Chronic spontaneous urticaria	Chronic spontaneous urticaria
Phase	Phase 3	Phase 2
Patients	800	226
Primary Outcome Measures	The proportion of subjects with well-controlled disease (UAS7 ≤ 6) at week 12	Long-term safety; number of participants with treatment-emergent adverse events
Arms Intervention	Ligelizumab Dose 1 and 3 Ligelizumab Dose 2 and 3	Ligelizumab 240 mg q4wks open label for 52 weeks
Target Patients	Patients who completed studies CQGE031C2302, CQGE031C2303, CQGE031C2202 or CQGE031C1301	Adult patients with chronic spontaneous urticaria inadequately controlled with H1-antihistamines at approved or increased doses, alone or in combination with H2-antihistamines or leukotriene receptor antagonists.
Read-out Milesstone(s)	2026	2019 (actual)
Publication	Study design presented at 2020 EAACI	Manuscript: Primary results extension trial (JAMA), H2 2021

QGE031 - Anti-IgE

Study	NCT03580356 PEARL 2 (CQGE031C2303)	NCT03580369 PEARL 1 (CQGE031C2302)
Indication	Chronic spontaneous urticaria	Chronic spontaneous urticaria
Phase	Phase 3	Phase 3
Patients	1050	1050
Primary Outcome Measures	Absolute change from baseline in UAS7 (Urticaria Activity Score) at week 12	Absolute change from baseline in UAS7 (Urticaria Activity Score) at week 12
Arms Intervention	Ligelizumab dose A q4w for 52 weeks Ligelizumab dose B q4w for 52 weeks Omalizumab 300 mg q4w for 52 weeks Placebo q4w from randomization to wk20, then ligelizumab dose B from wk24 to wk52	Ligelizumab dose A q4w for 52 weeks Ligelizumab dose B q4w for 52 weeks Omalizumab 300 mg q4w for 52 weeks Placebo q4w from randomization to wk20, then ligelizumab dose B from wk24 to wk52
Target Patients	Adolescents and adults with chronic spontaneous urticaria inadequately controlled with H1-antihistamines	Adolescents and adults with chronic spontaneous urticaria inadequately controlled with H1-antihistamines
Read-out Milesstone(s)	H2-2021 (Q4/2021-Q1/2022 potential COVID impact)	H2-2021 (Q4/2021-Q1/2022 potential COVID impact)
Publication	Past publications: Study design presented at UCARE 2018 Congress: primary results EADV 2022 (H2 2022) or AAAAI 2023 Manuscript: primary results, Journal (TBD), 2023	Past publications: Study design presented at UCARE 2018 Congress: primary results EADV 2022 (H2 2022) or AAAAI 2023 Manuscript: primary results, Journal (TBD), 2023

VAY736 - BAFF-R inhibitor

Study	NCT03217422 AMBER (CVAY736B2201)
Indication	Autoimmune hepatitis
Phase	Phase 2
Patients	80
Primary Outcome Measures	Alanine aminotransferase (ALT) normalization
Arms Intervention	VAY736 Placebo control with conversion to active VAY736
Target Patients	Autoimmune hepatitis patients with incomplete response or intolerant to standard treatment of care
Read-out Milesstone(s)	2026
Publication	TBD

Neuroscience

Aimovig® - CGRP receptor antagonist

Study	NCT03867201 DRAGON (CAMG334A2304)
Indication	Migraine
Phase	Phase 3
Patients	550
Primary Outcome Measures	Change from baseline in monthly migraine days during the last 4 weeks of the 12-week treatment period
Arms Intervention	Subcutaneous injection of AMG334 (erenumab) 70 mg Subcutaneous injection of placebo
Target Patients	Adult chronic migraine patients
Read-out Milesstone(s)	Double-blind FIR for 100% of pts 2021; Q4 2021 Extension (open-label): 2024
Publication	Planned in H2-2022 for double-blind phase and H1-2025 for open-label extension phase

LMI070 - SMN2 RNA splice modulator

Study	NCT02268552 (CLMI070X2201)
Indication	Type 1 spinal muscular atrophy
Phase	Phase 1/2
Patients	39
Primary Outcome Measures	Number of participants with adverse events (AEs) and serious adverse events (SAEs)
Arms Intervention	Branaplam oral, once weekly: Part 1: 5 ascending doses Part 2: 2 different dose levels Part 3: patients continue on initial dose assigned in Part 1 or Part 2
Target Patients	Patients with type 1 spinal muscular atrophy
Read-out Milesstone(s)	Study Part 2: Q3-2020 (actual) Study Part 3: 2023
Publication	TBD

MIJ821 - NR2B negative allosteric modulator (NAM)

Study	**NCT04722666 (CMIJ821A12201)**
Indication	Depression (Major Depressive Disorder)
Phase	Phase 2
Patients	195
Primary Outcome Measures	Change from baseline to 24 hours in the total score of the Montgomery Åsberg Depression Rating Scale (MADRS)
Arms Intervention	MIJ821 (mg/kg) very low dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) low dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) high dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) very high dose for 40 minutes IV infusion on Day 1, Day 15 and Day 29 Placebo 40 minutes IV infusion of 0.9% sodium chloride on Day 1, Day 15 and Day 29 MIJ821 (mg/kg) high dose for 40 minutes IV infusion on Day 1 or 0.9% sodium chloride for 40 minutes IV infusion MIJ821 (mg/kg) very high dose for 40 minutes IV infusion on Day 1 or 0.9% sodium chloride for 40 minutes IV infusion
Target Patients	Participants with major depressive disorder who have suicidal ideation with intent
Read-out Milesstone(s)	2023
Publication	TBD

OMB157 - Anti-CD20

Study	NCT03650114 ALITHIOS (COMB157G2399)
Indication	Multiple Sclerosis
Phase	Phase 3
Patients	2010
Primary Outcome Measures	Evaluate the long-term safety and tolerability of ofatumumab 20 mg subcutaneous (sc) once every 4 (q4) weeks in subjects with RMS from the first dose of ofatumumab
Arms Intervention	Ofatumumab 20 mg every 4 weeks
Target Patients	Patients with relapsing MS
Read-out Milesstone(s)	2028
Publication	TBD

Zolgensma® - SMN1 gene replacement therapy

Study	NCT03381729 STRONG (CL-102)	NCT03837184 STRIVE Asia Pacific (CL-306)
Indication	Type 2 spinal muscular atrophy	Type 1 spinal muscular atrophy
Phase	Phase 1	Phase 3
Patients	51	2
Primary Outcome Measures	Safety and tolerability, incidence of adverse events Proportion of patients achieving Standing Milestone Change in Hammersmith Functional Motor Scale	Proportion of participants sitting without support
Arms Intervention	Open-label, single-arm, single-dose, intrathecal	Open-label, single-arm, single-dose, intravenous
Target Patients	Patients with spinal muscular atrophy with 3 copies of SMN2	Patients with spinal muscular atrophy Type 1
Read-out Milesstone(s)	Cohort B: Q4-2019 (actual); Cohort C1: TBC	H2-2021
Publication	TBD	TBD

Zolgensma® - SMN1 gene replacement therapy

Study	NCT03505099 SPR1NT (CL-304)
Indication	Spinal muscular atrophy
Phase	Phase 3
Patients	30
Primary Outcome Measures	[2 copies of SMN2] Percentage of participants achieving functional independent sitting for at least 30 seconds at any visit [3 copies of SMN2] Percentage of participants achieving the ability to stand without support for at least 3 seconds at any visit
Arms Intervention	Open-label, single-arm, single-dose, intravenous
Target Patients	Pre-symptomatic patients with spinal muscular atrophy and multiple copies SMN2
Read-out Milesstone(s)	H2-2021 (3-copy cohort)
Publication	Final study results of 2-copy cohort as late-breaker oral presentation at EAN Jun 22 2021

Oncology

177Lu-PSMA-617 - Radioligand therapy targeting prostate specific membrane antigen (PSMA)

Study	NCT03511664 VISION (PSMA-617-01)
Indication	PSMA-positive Metastatic Castration-resistant Prostate Cancer (mCRPC)
Phase	Phase 3
Patients	831
Primary Outcome Measures	Radiographic Progression Free Survival Overall Survival
Arms Intervention	177Lu-PSMA-617 plus BS/BSC BS/BSC alone
Target Patients	Adult patients with PSMA-positive Metastatic Castration-resistant Prostate Cancer (mCRPC)
Read-out Milesstone(s)	Primary Analysis: Mar-2021 (Actual) Final Analysis: Q4-2022
Publication	Morris et al. Presented at ASCO (6-Jun-2021) Manuscript e-publication expected Jun-2021

177Lu-PSMA-617 - Radioligand therapy target PSMA

Study	NCT04689828 PSMAfore (CAAA617B12302)	NCT04720157 PSMAddition (CAAA617C12301)
Indication	Metastatic castration-resistant prostate cancer, pre-taxane	Metastatic hormone sensitive prostate cancer
Phase	Phase 3	Phase 3
Patients	495	1126
Primary Outcome Measures	Radiographic Progression Free Survival (rPFS)	Radiographic Progression Free Survival (rPFS)
Arms Intervention	Arm 1: Participants will receive 7.4 GBq (200 mCi) +/- 10% 177Lu-PSMA-617 once every 6 weeks for 6 cycles. Best supportive care, including ADT may be used Arm 2: For participants randomized to the ARDT arm, the change of ARDT treatment will be administered per the physician's orders. Best supportive care, including ADT may be used	Arm 1: 177Lu-PSMA-617 Participant will receive 7.4 GBq (+/- 10%) 177Lu-PSMA-617, once every 6 weeks (+/- 1 week) for a planned 6 cycles, in addition to the Standard of Care (SOC); ARDT +ADT is considered as SOC and treatment will be administered per the physician's order Arm 2: For participants randomized to Standard of Care arm, ARDT +ADT is considered as SOC and treatment will be administered per the physician's order
Target Patients	mCRPC patients that were previously treated with an alternate ARDT and not exposed to a taxane-containing regimen in the CRPC or mHSPC settings	Patients with metastatic Hormone Sensitive Prostate Cancer (mHSPC)
Read-out Milesstone(s)	Primary Analysis: 2022 Final Analysis: 2025	Final Analysis: 2024
Publication	TBD	TBD

ABL001 - Specific, allosteric Bcr-Abl kinase inhibitor

Study	NCT03106779 ASCEMBL (CABL001A2301)
Indication	Chronic myeloid leukaemia (CML)
Phase	Phase 3
Patients	233
Primary Outcome Measures	Major Molecular Response (MMR) rate at 24 weeks
Arms Intervention	ABL001 40 mg bid Bosutinib 500 mg
Target Patients	Patients with chronic myelogenous leukemia in chronic phase, previously treated with 2 or more tyrosine kinase inhibitors
Read-out Milesstone(s)	Q3-2020 (actual)
Publication	Hochhaus A., et al. [Efficacy and Safety Results from ASCEMBL, a Multicenter, Open-Label, Phase 3 Study of Asciminib, a First-in-Class STAMP Inhibitor, vs Bosutinib (BOS) in Patients (Pts) with Chronic Myeloid Leukemia in Chronic Phase (CML-CP) Previously Treated with ≥2 Tyrosine Kinase Inhibitors (TKIs), LBA-4] ASH 2020 Manuscript submission H1-2021

ACZ885 - IL-1beta inhibitor

Study	NCT03447769 CANOPY-A (CACZ885T2301)	NCT03626545 CANOPY-2 (CACZ885V2301)
Indication	Adjuvant NSCLC	2nd / 3rd Line Non-small cell lung cancer (NSCLC)
Phase	Phase 3	Phase 3
Patients	1500	240
Primary Outcome Measures	Disease free survival (primary), overall survival (key secondary)	Safety run-in part: Incidence of dose limiting toxicities Double-blind, randomized, placebo-controlled part: Overall Survival
Arms Intervention	Canakinumab 200mg q3w sc for 18 cycles Placebo q3w sc for 18 cycles	Canakinumab in combination with docetaxel Canakinumab matching-placebo in combination with docetaxel
Target Patients	Patients with: High-risk NSCLC (AJCC/UICC v.8 stage II-IIIA and IIIB (T>5cm N2)) after complete resection and standard of care adjuvant cisplatin-based chemotherapy All histologies	Patients with: Stage IIIB or IV NSCLC without EGFR, ALK, ROS-1 or B-RAF mutation Previously treated with platinum therapy and PD(L)1-inhibitor
Read-out Milesstone(s)	2023	March-2021 (Actual)
Publication	TBD	Abstract submitted to ESMO, manuscript in progress.

ACZ885 - IL-1beta inhibitor

Study	NCT03631199 CANOPY-1 (CACZ885U2301)
Indication	1st Line Non-small cell lung cancer (NSCLC)
Phase	Phase 3
Patients	627
Primary Outcome Measures	Safety run-in part: Incidence of dose limiting toxicities Double-blind, randomized, placebo-controlled part: Progression free survival (PFS) Overall survival (OS)
Arms Intervention	Canakinumab or matching placebo in combination with pembrolizumab and platinum-based doublet chemotherapy
Target Patients	Patients with Histologically confirmed Stage IIIB, IV NSCLC with no prior systemic anticancer therapy Squamous and non-squamous NSCLC No EGFR mutation and ALK rearrangement
Read-out Milesstone(s)	H2-2021
Publication	Johnson B et al. Presented at AACR-NCI-EORTC 2019 (safety run-in) Planned abstract submission to congress in 2H 2021

BYL719 - Alpha-specific PI3K inhibitor

Study	NCT04208178 EPIK-B2 (CBYL719G12301)	NCT04251533 EPIK-B3 (CBYL719H12301)
Indication	HER-2 positive breast cancer	Triple negative breast cancer
Phase	Phase 3	Phase 3
Patients	548	566
Primary Outcome Measures	Progression-free survival (PFS)	Progression-free Survival (PFS) for patients with PIK3CA mutant status
Arms Intervention	Alpelisib + trastuzumab + pertuzumab Trastuzumab + pertuzumab	Alpelisib 300 mg + nab-paclitaxel 100 mg/m² Placebo + nab-paclitaxel 100 mg/m²
Target Patients	Patients with HER2-positive advanced breast cancer with a PIK3CA mutation	Patients with advanced triple negative breast cancer with either Phosphoinositide-3-kinase Catalytic Subunit Alpha (PIK3CA) mutation or Phosphatase and Tensin Homolog Protein (PTEN) loss without PIK3CA mutation
Read-out Milesstone(s)	2025	2023
Publication	TBD	TBD

BYL719 - Alpha-specific PI3K inhibitor

Study	NCT04589650 EPIK-P2 (CBYL719F12201)
Indication	PIK3CA-related overgrowth spectrum
Phase	Phase 2
Patients	150
Primary Outcome Measures	Proportion of participants with a response at Week 24
Arms Intervention	Arm 1: alpelisib vs. Arm 2: placebo during the 16 first weeks. for each cohort (adult, pediatric)
Target Patients	Pediatric and adult participants with PIK3CA-related overgrowth spectrum (PROS)
Read-out Milesstone(s)	Primary Analysis: H1-2023
Publication	NA

Jakavi® - JAK1/2 inhibitor

Study	NCT03491215 REACH4 (CINC424F12201)	NCT03774082 REACH5 (CINC424G12201)
Indication	Acute graft versus host disease	Chronic graft versus host disease
Phase	Phase 2	Phase 2
Patients	45	42
Primary Outcome Measures	Measurement of PK parameters Overall Response Rate (ORR)	Overall Response Rate (ORR)
Arms Intervention	Ruxolitinib	Ruxolitinib 5mg tablets / pediatric formulation
Target Patients	Pediatric patients with grade II-IV acute graft vs. host disease after allogeneic hematopoietic stem cell transplantation	Pediatric subjects with moderate and severe chronic Graft vs. Host disease after allogeneic stem cell transplantation
Read-out Milesstone(s)	2023	2023
Publication	TBD	TBD

Jakavi® - JAK1/2 inhibitor

Study	NCT04097821 ADORE (CINC424H12201)
Indication	Myelofibrosis
Phase	Phase 1/2
Patients	130
Primary Outcome Measures	Incidence of dose limiting toxicities within the first 2 cycles Response rate at the end of cycle 6
Arms Intervention	Ruxolitinib Ruxolitinib+Siremadlin Ruxolitinib+Crizanlizumab Ruxolitinib+MBG453 Ruxolitinib+LTT462 Ruxolitinib+NIS793
Target Patients	Patients with Myelofibrosis (MF)
Read-out Milesstone(s)	2024
Publication	TBD

Kisqali® - CDK 4/6 inhibitor

Study	NCT03701334 NATALEE (CLEE011O12301C)
Indication	Adjuvant treatment of hormone receptor (HR)-positive, HER2-negative, early breast cancer (EBC)
Phase	Phase 3
Patients	
Primary Outcome Measures	Invasive Disease-Free Survival for using STEEP criteria (Standardized Definitions for Efficacy End Points in adjuvant breast cancer trials)
Arms Intervention	Ribociclib + endocrine therapy Endocrine therapy
Target Patients	Pre and postmenopausal women and men with HR-positive, HER2-negative EBC, after adequate surgical resection, who are eligible for adjuvant endocrine therapy
Read-out Milesstone(s)	2022
Publication	TBD

Piqray® - PI3K-alpha inhibitor

Study	NCT04729387 EPIK-O (CBYL719K12301)
Indication	Ovarian Cancer
Phase	Phase 3
Patients	
Primary Outcome Measures	Progression Free Survival (PFS) based on Blinded Independent Review Committee (BIRC) assessment using RECIST 1.1 criteria
Arms Intervention	Arm 1 Experimental: Alpelisib+olaparib: Alpelisib 200 mg orally once daily and olaparib 200 mg orally twice daily on a continuous dosing schedule Arm 2 Active Comparator: Paclitaxel or PLD. Investigator's choice of one of 2 single agent cytotoxic chemotherapies: Paclitaxel 80 mg/m2 intravenously weekly or Pegylated liposomal Doxorubicin (PLD) 40-50 mg/m2 (physician discretion) intravenously every 28 days.
Target Patients	Patients with platinum resistant or refractory high-grade serous ovarian cancer, with no germline BRCA mutation detected
Read-out Milesstone(s)	2023
Publication	TBD

Kymriah® - CAR-T therapy

Study	NCT03568461 ELARA (CCTL019E2202)	NCT03570892 BELINDA (CCTL019H2301)
Indication	Relapsed / refractory follicular lymphoma (FL)	2nd line Diffuse large B-cell lymphoma (DLBCL)
Phase	Phase 2	Phase 3
Patients	97	318
Primary Outcome Measures	Complete Response Rate (CRR)	Event-free Survival (EFS)
Arms Intervention	Single-arm study of tisagenlecleucel	Tisagenlecleucel versus standard of care
Target Patients	Adult patients with relapsed or refractory FL	Adult patients with aggressive B-cell Non-Hodgkin Lymphoma after failure of rituximab and anthracycline- containing frontline immunochemotherapy
Read-out Milesstone(s)	H1-2021 (actual)	H2-2021
Publication	Schuster, et al. presented at ASCO, EHA and ICML 2021	Bishop et al at SITC 2019 Abstract submission to congress in H2-2021

Kymriah® - CAR-T therapy

Study	NCT03876769 CASSIOPEIA (CCTL019G2201J)
Indication	1st line high risk acute lymphoblastic leukemia (ALL)
Phase	Phase 2
Patients	160
Primary Outcome Measures	Disease Free Survival (DFS)
Arms Intervention	Single-arm study of tisagenlecleucel
Target Patients	Pediatric and young adult patients with 1st line high risk ALL
Read-out Milesstone(s)	2025
Publication	TBD

MBG453 - TIM-3 antagonist

Study	NCT03946670 STIMULUS MDS-1 (CMBG453B12201)	NCT04266301 STIMULUS-MDS2 (CMBG453B12301)
Indication	Myelodysplastic syndrome	Myelodysplastic syndrome
Phase	Phase 2	Phase 3
Patients	120	500
Primary Outcome Measures	Complete Remission (CR) rate and Progression Free Survival (PFS)	Overall survival
Arms Intervention	Experimental: Sabatolimab (MBG453) + hypomethylating agents Placebo comparator: Placebo + hypomethylating agents	Sabatolimab 800 mg + azacitidine 75 mg/m2 Sabatolimab 800 mg + azacitidine 75 mg/m2 + placebo
Target Patients	Adult subjects with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as per IPSS-R criteria	Patients with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as Per IPSS-R, or Chronic Myelomonocytic Leukemia-2 (CMML-2)
Read-out Milesstone(s)	2022-2023	2023
Publication	TBD	TBD

MBG453 - TIM-3 antagonist

Study	NCT04150029 STIMULUS-AML1 (CMBG453C12201)
Indication	Unfit acute myeloid leukaemia
Phase	Phase 2
Patients	86
Primary Outcome Measures	Incidence of dose limiting toxicities (Safety run-in patients only) Percentage of subjects achieving complete remission (CR)
Arms Intervention	Single arm safety and efficacy study of sabatolimab in combination with azacitidine and venetoclax
Target Patients	Newly diagnosed adult AML patients who are not suitable for treatment with intensive chemotherapy
Read-out Milesstone(s)	2023
Publication	TBD

NIS793 - TGFβ1 inhibitor

Study	**NCT02947165 (CNIS793X2101)**
Indication	Solid tumors
Phase	Phase 1
Patients	120
Primary Outcome Measures	Incidence of DLTs, AEs, SAEs and dose reductions / interruptions for NIS793 Incidence of DLTs, AEs, SAEs and dose reductions/interruptions for NIS793 in combination with PDR001
Arms Intervention	NIS793 NIS793 + PDR001
Target Patients	Adult patients with advanced malignancies
Read-out Milesstone(s)	2021
Publication	TBD

PDR001 - PD-1 checkpoint inhibitor

Study	**NCT03484923 (CPDR001J2201)**
Indication	Previously treated unresectable or metastatic melanoma
Phase	Phase 2
Patients	195
Primary Outcome Measures	Objective Response Rate (ORR)
Arms Intervention	Spartalizumab (PDR001) 400mg i.v. Q4W + LAG525 (to be tested in unselected patients and LAG-3 positive patients) Spartalizumab 400mg i.v. Q4W + capmatinib Spartalizumab 400mg i.v. Q4W + canakinumab Spartalizumab 400mg i.v. Q4W + ribociclib
Target Patients	Adult patients with previously treated unresectable or metastatic melanoma
Read-out Milesstone(s)	2022
Publication	TBD

Promacta®/Revolade® - Thrombopoetin receptor agonist

Study	NCT03025698 (CETB115E2201)	NCT03988608 (CETB115E2202)
Indication	Previously untreated or relapsed/refractory severe aplastic anemia or recurrent aplastic anemia	Previously untreated or relapsed/refractory severe aplastic anemia or recurrent aplastic anemia
Phase	Phase 2	Phase 2
Patients	60	20
Primary Outcome Measures	PK of eltrombopag at steady state in pediatric patients with SAA	Hematologic response rate rate up to 26 weeks of treatment
Arms Intervention	Eltrombopag 12.5, 25, 50, 75 mg FCT & 25 mg pFOS Arm A: relapsed/refractory SAA or recurrent AA following IST for SAA: hATG/cyclosporine + eltrombopag or cyclosporine + eltrombopag Arm B: previously untreated SAA: hATG/cyclosporine + eltrombopag	Eltrombopag 25 mg film-coated tablets
Target Patients	Pediatric patients from age 1 <18 years with relapsed/refractory SAA or recurrent AA after IST or previously untreated SAA	Chinese patients with refractory or relapsed severe aplastic anemia
Read-out Milesstone(s)	Primary CSR: 2022 Final CSR: 2025	Primary: 2021; Final: 2023
Publication	TBD	TBD

Rydapt® - Multi-targeted kinase inhibitor

Study	NCT03591510 (CPKC412A2218)
Indication	Acute myeloid leukemia
Phase	Phase 2
Patients	50
Primary Outcome Measures	Occurrence of dose limiting toxicities Event Free Survival (EFS)
Arms Intervention	Chemotherapy followed by Midostaurin
Target Patients	Newly diagnosed pediatric patients with FLT3 mutated acute myeloid leukemia (AML)
Read-out Milesstone(s)	2025
Publication	TBD

SEG101 - p-Selectin inhibitor

Study	NCT03474965 SOLACE-Kids (CSEG101B2201)	NCT03814746 STAND (CSEG101A2301)
Indication	Prevention of VOC in pediatric patients with SCD	Prevention of Vaso-Occlusive Crises (VOC) in patients with Sickle Cell Disease (SCD)
Phase	Phase 2	Phase 3
Patients	100	240
Primary Outcome Measures	PK/PD and safety of SEG101 at 5 mg/kg	Rate of VOC events leading to healthcare visit
Arms Intervention	SEG101 (crizanlizumab) at a dose of 5 mg/kg by IV infusion ± Hydroxyurea/Hydroxycarbamide	Crizanlizumab 5.0 mg/kg Crizanlizumab 7.5 mg/kg Placebo
Target Patients	Pediatric SCD patients with VOC	Adolescent and adult SCD patients (12 years and older)
Read-out Milesstone(s)	H2-2021 (pediatric patients ≥12 year old) 2024 (pediatric patients <12 year old)	2022
Publication	Planned abstract submission to congress in H2-2021	TBD

Tabrecta® - Met Inhibitor

Study	NCT04427072 (CINC280A2301)
Indication	Non-small cell lung cancer
Phase	Phase 3
Patients	90
Primary Outcome Measures	Progression free survival (PFS) per blinded independent review committee (BIRC) using RECIST v1.1
Arms Intervention	Arm 1: 400mg of capmatinib tablets administered orally twice daily Arm 2: Docetaxel 75 mg/m2 by intravenous infusion every 21 days
Target Patients	Previously Treated Patients With EGFR wt, ALK Negative, Locally Advanced or Metastatic (Stage IIIB/IIIC or IV) NSCLC Harboring MET Exon 14 Skipping Mutation (METΔex14).
Read-out Milesstone(s)	Primary 2022 Final: 2024
Publication	TBD

Tafinlar® - BRAF inhibitor

Study	NCT01677741 (CDRB436A2102)
Indication	BRAFV600 mutant cancers
Phase	Phase 1/2
Patients	85
Primary Outcome Measures	Safety, tolerability and pharmacokinetics
Arms Intervention	Single-arm study of oral dabrafenib (dose based on age and weight)
Target Patients	Pediatric subjects aged 1 year to <18 years with advanced BRAF V600-mutation positive solid tumors
Read-out Milesstone(s)	H1-2021 (actual)
Publication	Kieran MW et al. Clin Cancer Res 2019;25(24):7294-7302 (PK analysis) Hargrave DR et al. Clin Cancer Res 2019;25(24):7303-7311 (safety/efficacy in low-grade gliomas)

Tafinlar®+Mekinist® - BRAF inhibitor and MEK inhibitor

Study	NCT02684058 (CDRB436G2201)
Indication	BRAFV600 mutant gliomas
Phase	Phase 2
Patients	142
Primary Outcome Measures	Objective response rate
Arms Intervention	Dabrafenib + trametinib (dose based on age and weight)
Target Patients	Children and adolescent patients with BRAF V600 mutation positive relapsed or refractory high grade glioma (HGG) or BRAF V600 mutation positive low grade glioma (LGG)
Read-out Milesstone(s)	2022
Publication	TBD

TNO155 - SHP2 Inhibitor

Study	NCT03114319 (CTNO155X2101)	NCT04000529 (CTNO155B12101)
Indication	Solid tumors (single agent)	Solid tumors (combo)
Phase	Phase 1	Phase 1
Patients	255	126
Primary Outcome Measures	Number of participants with adverse events Number of participants with dose limiting toxicities	Incidence of dose limiting toxicities (DLTs) during the first cycle of combination treatment during the dose escalation part Incidence and severity of adverse events (AEs) and serious adverse events (SAEs) as per CTCAE v5.0, by treatment Dose tolerability
Arms Intervention	Drug: TNO155 Drug: TNO155 in combination with EGF816 (nazartinib)	TNO155 and Spartalizumab (PDR001) TNO155 and Ribociclib (LEE011)
Target Patients	Adult patients with advanced solid tumors in selected indications	Patients with advanced malignancies
Read-out Milesstone(s)	2023	2022
Publication	TBD	TBD

Ophthalmology

Beovu® - Anti-VEGF

Study	NCT03386474 (CRTH258A2301E1)	NCT03481634 KESTREL (CRTH258B2301)
Indication	Neovascular age-related macular degeneration (nAMD)	Diabetic eye disease
Phase	Phase 3	Phase 3
Patients	150	534
Primary Outcome Measures	Number of treatment-emergent adverse events	Change from baseline in best-corrected visual acuity (BCVA)
Arms Intervention	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL	Brolucizumab (RTH258) 3 mg/50 µL Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2mg/50 uL
Target Patients	Patients with neovascular age-related macular degeneration who have completed the CRTH258A2301 study	Patients with visual impairment due to diabetic macular edema (DME)
Read-out Milesstone(s)	2018 (actual)	Primary: Q4-2020 (actual); Final: H2-2021
Publication	Planned publication of the attributes of brolucizumab and durability in H1-2021	Brown et al., presented at ARVO May 2021 Manuscript submission H2 2021

Beovu® - Anti-VEGF

Study	NCT03481660 KITE (CRTH258B2302)	NCT03917472 KINGFISHER (CRTH258B2305)
Indication	Diabetic eye disease	Diabetic macular edema
Phase	Phase 3	Phase 3
Patients	356	500
Primary Outcome Measures	Change from baseline in best-corrected visual acuity (BCVA)	Change in best-corrected visual acuity (BCVA) from baseline up to week 52
Arms Intervention	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL
Target Patients	Patients with visual impairment due to diabetic macular edema (DME)	Patients with visual impairment due to diabetic macular edema
Read-out Milesstone(s)	Primary: Q3-2020 (actual); Final: H2-2021	H2-2021
Publication	Brown et al., presented at ARVO May 2021 Manuscript submission H2 2021	TBC

Beovu® - Anti-VEGF

Study	NCT04005352 TALON (CRTH258A2303)	NCT04047472 HOBBY (CRTH258A2307)
Indication	Neovascular Age-related Macular Degeneration (nAMD)	Macular degeneration
Phase	Phase 3B	Phase 3
Patients		494
Primary Outcome Measures	Average change in Best-corrected visual acuity Distribution of the last interval with no disease activity (in a Treat-to-Control regimen)	Change from baseline in best-corrected visual acuity (BCVA) at week 48
Arms Intervention	Arm 1: Brolucizumab 6 mg intravitreal injection Arm 2: Aflibercept 2 mg intravitreal injection	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL
Target Patients	Patients with Neovascular Age-related Macular Degeneration (nAMD) who have not previously received anti-VEGF (vascular endothelial growth factor) treatment	Chinese patients with neovascular age-related macular degeneration
Read-out Milesstone(s)	2022	2024
Publication	TBD	TBD

Beovu® - Anti-VEGF

Study	NCT04058067 KINGLET (CRTH258B2304)	NCT04278417 (CRTH258D2301)
Indication	Diabetic macular edema	Diabetic retinopathy
Phase	Phase 3	Phase 3
Patients	268	706
Primary Outcome Measures	Change in best-corrected visual acuity (BCVA)	Change from Baseline in BCVA
Arms Intervention	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL	Arm 1: RTH258 (Brolucizumab) 6 mg3 x q6w loading injections, followed by q12w maintenance through week 90 Arm 2: Panretinal photocoagulation laser initial treatment in 1-4 sessions up to Week 12, followed with additional PRP treatment as needed
Target Patients	Chinese patients with visual impairment due to diabetic macular edema	Patients with proliferative diabetic retinopathy
Read-out Milesstone(s)	2023	2024
Publication	Publication planned for 2023	TBD

ECF843 - rh-Lubricin

Study	**NCT04391894 (CECF843A2201)**
Indication	Dry eye
Phase	Phase 2
Patients	680
Primary Outcome Measures	Change from baseline in symptom assessment in Dry Eye (SANDE) score Change from baseline in composite corneal fluorescein staining score
Arms Intervention	A Study to Assess the Safety and Efficacy of ECF843 vs Vehicle in Subjects with dry eye disease ECF843 0.15 or 0.45 mg/mL BID/TID/vehicle
Target Patients	Patients with moderate to severe dry eye disease (DED)
Read-out Milesstone(s)	H2-2021 (actual)
Publication	TBD

Lucentis® - Anti-VEGF

Study	NCT02640664 RAINBOW Extension (CRFB002H2301E1)
Indication	Retinopathy of Prematurity (ROP)
Phase	Phase 3
Patients	180
Primary Outcome Measures	To evaluate the visual function of patients by assessing the visual acuity in the better-seeing eye at the patient's fifth birthday.
Arms Intervention	Ranibizumab 0.2 mg (up to Week 40, if warranted) Ranibizumab 0.1 mg (up to Week 40, if warranted)
Target Patients	Male and female preterm infants with bilateral retinopathy of prematurity (ROP) who completed RAINBOW.
Read-out Milesstone(s)	2023
Publication	TBD

SAF312 - TRPV1 antagonist

Study	NCT04630158 SAHARA (CSAF312B12201)
Indication	Chronic ocular surface pain
Phase	Phase 2
Patients	150
Primary Outcome Measures	Change in mean pain severity Visual Analog Scale
Arms Intervention	Placebo Comparator: SAF312 Placebo. Randomized to a 1:1:1 topical eye drops, twice daily Experimental: SAF312 dose 1. Randomized to a 1:1:1 topical eye drops, twice daily Experimental: SAF312 dose 2. Randomized to a 1:1:1 topical eye drops, twice daily
Target Patients	Subjects with CICP persisting at least for 4 months after refractive surgery and chronicity confirmed during the observational period.
Read-out Milesstone(s)	2022
Publication	TBD

UNR844 - Reduction of disulfide bonds

Study	READER (CUNR844A2022)
Indication	Presbyopia
Phase	Phase 2B
Patients	225
Primary Outcome Measures	Characterize the dose response relationship among UNR844 doses 0 mg/mL, 5 mg/mL, 13.3 mg/mL, 23 mg/mL and 30 mg/mL dosed twice-daily after Month 3 of dosing. Change from baseline in Binocular distance-corrected near visual acuity at 40 cm at Month 3.
Arms Intervention	1:1 randomization - UNR844 0 mg/mL, 5 mg/mL, 13.3 mg/mL, 23 mg/mL and 30 mg/mL dosed twice-daily for three months
Target Patients	Presbyopic participants aged 45 to 55 years
Read-out Milesstone(s)	Q3 2022: Interim analysis (Primary endpoint) - when all patients have completed the 3 months treatment period Q4 2022: Interim analysis - when 60% of patients have completed 6 months of post treatment follow-up Q2 2023: Final analysis -Study completion (all patients have completed 9 months pots treatment period)
Publication	TBD

Respiratory

CSJ117 - TSLP inhibitor

Study	NCT04410523 (CCSJ117A12201C)
Indication	Asthma
Phase	Phase 2
Patients	625
Primary Outcome Measures	Pre-dose FEV1 (Forced Expiratory Volume in 1 second) change from baseline after 12 weeks of treatment. Average change from baseline in pre-dose FEV1 at week 8 & week 12
Arms Intervention	CSJ117 0.5mg CSJ117 1mg CSJ117 2 mg CSJ117 4 mg CSJ117 8 mg Placebo
Target Patients	Asthma patients on background medium or high ICS plus LABA therapy
Read-out Milesstone(s)	2022
Publication	Primary publications planned for 2022

QBW251 - CFTR potentiator

Study	NCT04072887 (CQBW251B2201)
Indication	Chronic obstructive pulmonary disease (COPD)
Phase	Phase 2
Patients	956
Primary Outcome Measures	Trough FEV1 (Forced Expiratory Volume in 1 second) change from baseline after 12 weeks of treatment
Arms Intervention	QBW251 450 mg QBW251 300 mg QBW251 150 mg QBW251 75 mg QBW251 25 mg Placebo
Target Patients	COPD patients on background triple inhaled therapy (LABA / LAMA / ICS)
Read-out Milesstone(s)	H1-2022
Publication	Primary publications planned for 2022

Sandoz Biopharmaceuticals

GP2411 - Biosimilar denosumab

Study	**NCT03974100 (CGP24112301)**
Indication	Osteoporosis
Phase	Phase 3
Patients	522
Primary Outcome Measures	Percent change from baseline (%CfB) in lumbar spine Bone Mineral Density
Arms Intervention	GP2411 60 mg /mL subcutaneous injection every 6 months Prolia® 60 mg /mL subcutaneous injection every 6 months
Target Patients	Postmenopausal women with osteoporosis
Read-out Milesstone(s)	2022
Publication	Study data publications expected for 2024 and beyond. The overall study design will be published at WCO and ECTS congresses 2020.

Global Health

KAF156 - Plasmodium Falciparum Inhibitor - PfCARL mediated

Study	**NCT03167242 (CKAF156A2202)**
Indication	Malaria
Phase	Phase 2
Patients	
Primary Outcome Measures	PCR-corrected adequate clinical and parasitological response (ACPR)
Arms Intervention	KAF156 and LUM-SDF (different combinations) Coartem
Target Patients	Adults and children with uncomplicated Plasmodium Falciparum Malaria
Read-out Milesstone(s)	H2-2021
Publication	Two posters accepted, ASTMH meeting Nov 15-19 2020 Kublin JG et al. Clinical Infectious Diseases 09 Jul 2020, PMID: 32644127

artemether + lumefantrine - PGH-1

Study	NCT04300309 CALINA (CCOA566B2307)
Indication	Malaria, uncomplicated (<5kg patients)
Phase	Phase 3
Patients	
Primary Outcome Measures	Artemether Cmax
Arms Intervention	Experimental: artemether lumefantrine (2.5 mg:30 mg) artemether lumefantrine (2.5 mg:30 mg) bid over 3 days, from 1-4 tablets per dose
Target Patients	Infants and Neonates <5 kg body weight with acute uncomplicated plasmodium falciparum malaria
Read-out Milesstone(s)	Primary outcome measure: 2023
Publication	TBD

ganaplacide - Imidazolopiperazines derivative

Study	NCT04546633 KALUMI (CKAF156A2203)
Indication	Malaria, uncomplicated
Phase	Phase 2
Patients	
Primary Outcome Measures	PCR-corrected and uncorrected Adequate Clinical and Parasitological Response (ACPR)
Arms Intervention	KAF156 and LUM-SDF QD (once daily) for 2 days in fasted condition KAF156 and LUM-SDF QD (once daily) for 2 days in fed condition
Target Patients	Malaria patients 12 to < 18 years old with malaria caused by P. falciparum
Read-out Milesstone(s)	H1-2022
Publication	TBD

Abbreviations

aBC	Advanced breast cancer
AD	Atopic Dermatitis
AIH	Autoimmune hepatitis
aHUS	atypical Hemolytic Uremic Syndrome
ALL	Acute lymphoblastic leukemia
ALS	Amyotrophic lateral sclerosis
AMI	Acute myocardial infarction
AML	Acute myeloid leukemia
AS H2H	Ankylosing spondylitis head-to-head study versus adalimumab
BC	Breast cancer
C3G	C3 glomerulopathy
CCF	Congestive cardiac failure
CLL	Chronic lymphocytic leukemia
CML	Chronic myeloid leukemia
CRC	Colorectal cancer
COPD	Chronic obstructive pulmonary disease
COSP	Chronic ocular surface pain
CRSwNP	Severe chronic rhinosinusitis with nasal polyps
CSU	Chronic spontaneous urticaria
CVRR-Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein (a)
CVRR-LDLC	Secondary prevention of cardiovascular events in patients with elevated levels of LDLC
DME	Diabetic macular edema
DLBCL	Diffuse large B-cell lymphoma refractory
FL	Follicular lymphoma
GCA	Giant cell arteritis
GVHD	Graft-versus-host disease
HCC	Hepatocellular carcinoma
HD	Huntington's disease
HFpEF	Chronic heart failure with preserved ejection fraction
HF-rEF	Chronic heart failure with reduced ejection fraction
HNSCC	Head and neck squamous cell carcinoma
HS	Hidradenitis suppurativa
IA	Interim analysis
IgAN	IgA nephropathy
iMN	Membranous nephropathy
IPF	Idiopathic pulmonary fibrosis
JIA	Juvenile idiopathic arthritis
LVEF	Left ventricular ejection fraction
mCRPC	Metastatic castration-resistant prostate cancer
MDR	Multi-drug resistant
MDS	Myelodysplastic syndrome
MS	Multiple sclerosis
wAMD	Wet (neovascular) age-related macular degeneration
NASH	Non-alcoholic steatohepatitis
nHCM	Non-obstructive hypertrophic cardiomyopathy
nr-axSpA	Non-radiographic axial spondyloarthritis
NSCLC	Non-small cell lung cancer
PDR	Proliferative diabetic retinopathy
PEF	Preserved ejection fraction
PedPsO	Pediatric psoriasis
PNH	Paroxysmal nocturnal haemoglobinuria
PsA	Psoriatic arthritis
RCC	Renal cell carcinoma
PROS	PIK3CA related overgrowth spectrum
RA	Rheumatoid arthritis
rMS	Relapsing multiple sclerosis
ROP	Retinopathy of prematurity
RP	Retinitis pigmentosa
RVO	Retinal vein occlusion
SAA	Severe aplastic anemia
SLE	Systemic lupus erythematosus
SMA Type 1	Spinal muscular atrophy (IV formulation)
SMA Type 2/3	Spinal muscular atrophy (IT formulation)
SpA	Spondyloarthritis
SPMS	Secondary progressive multiple sclerosis
TNBC	Triple negative breast cancer
T1DM	Type 1 Diabetes mellitus