Q3 2022 Results
Investor presentation

NOVARTIS | Reimagining Medicine

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Vas Narasimhan

Chief Executive Officer

Company overview

Novartis delivers solid Q3 performance across our value drivers

Growth, cc 1

Group sales Q3 **+4%** (YTD +5%)

IM sales Q3 **+4%** (YTD +5%); **US** IM sales Q3 **+8%**

Sandoz sales Q3 **+4%** (YTD +6%)

Innovation 3

Scemblix approved in EU for Ph+ chronic myeloid leukemia

Pluvicto CHMP positive opinion for mCRPC post-taxane[3]

Iptacopan Ph3 PNH, clinically meaningful superiority vs anti-C5[3]

Cosentyx positive Ph3 SUNSHINE/SUNRISE in Hidradenitis Suppurativa

Productivity, cc 2

Group core operating income Q3 **+5%** (YTD +6%)

IM core operating income Q3 **+7%** (YTD +6%)

IM core margin Q3 38.1%, **+1.0%**pts (YTD 37.1%)

Sandoz core operating income Q3 **-5%** (YTD +5%)

SG&A savings of ~USD 1.5bn to be fully embedded by 2024[2]

ESG 4

Ganaplacide/lumefantrine Malaria[1] US FDA Orphan Drug and Fast Track Designation

Pediatric formulation of Hydroxyurea SCD launched in Ghana

Constant currencies (cc), core results are non-IFRS measures; explanation can be found on page 49 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY. IM – Innovative Medicines Division SCD – Sickle cell disease 1. Combination, being co-developed with Medicines for Malaria Venture, supported by EDCTP WANECAM2, for acute, uncomplicated malaria 2. Relating to streamlined organizational model. 3. Oct 2022

Strong performance of Entresto®, Kesimpta®, Kisqali ®, Pluvicto®

Q3 sales[1]

	Sales USD million	Growth vs. PY USD million	Growth vs. PY cc
Entresto® sacubitril/valsartan	1,135	211	31%
Kesimpta® (ofatumumab)	289	180	172%
KISQALI® ribociclib	327	95	49%
PLUVICTO™	80	80	nm
SCEMBLIX® (asciminib)	41	41	nm
Tafinlar® + Mekinist®	450	33	16%
LEQVIO®	34	29	nm
Cosentyx® (secukinumab)	1,274	27	7%
PIQRAY® (alpelisib) tablets	103	21	26%
MAYZENT® (siponimod) tablets	94	18	29%
LUTATHERA®	132	12	15%

Constant currencies (cc) is a non-IFRS measure; explanation of non-IFRS measures can be found on page 49 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.
nm – not meaningful 1. Innovative Medicines division.

Six in-market growth drivers with multi-bn sales potential and recent launches reinforce our confidence in mid-term growth outlook

6 in-market growth drivers, multi-bn potential

Cosentyx® | Entresto® | zolgensma®

KISQALI® | Kesimpta® | LEQVIO®

> 33% of IM sales growing 23% (Q3)

Recent launches

SCEMBLIX® | PLUVICTO™

> Scemblix and Pluvicto off to a good start

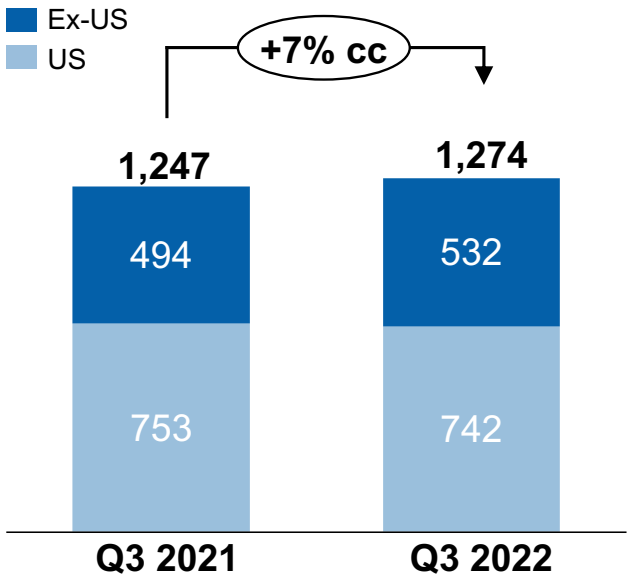
All growth rates in constant currencies (cc).

Cosentyx® showed steady growth, preparing for LCM

Cosentyx® | E | Z | K | K | L

Sales evolution

USD m, % cc



Maintaining competitive position across geographies

- >875k patients treated across 5 indications since launch
- US: Solid volume growth, offset by revenue deductions
- Europe: Leading originator biologic in PsO and SpA

Future growth drivers

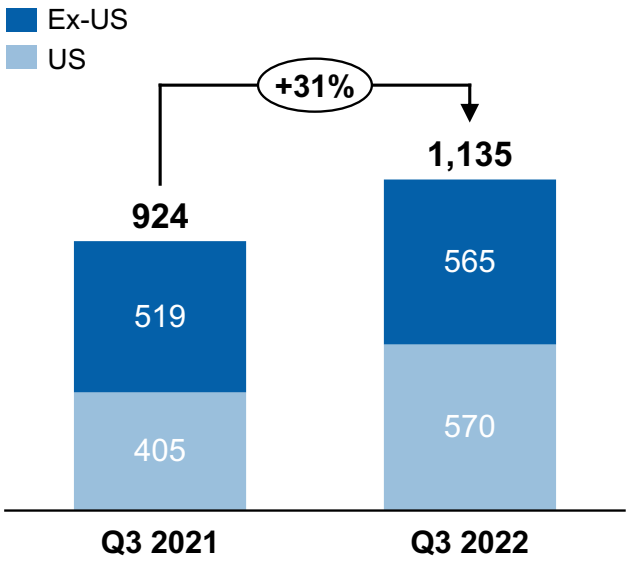
- Volume-driven growth across core indications
- Expanding geographical reach including China
- Hidradenitis Suppurativa regulatory file submitted to FDA and EMA
- IV regulatory file expected to be submitted in Q4 to FDA
- Additional life cycle management including giant cell arteritis

PsO – Psoriasis SpA – Spondyloarthritis IV – intravenous

Entresto® +31% cc, growing strongly across geographies

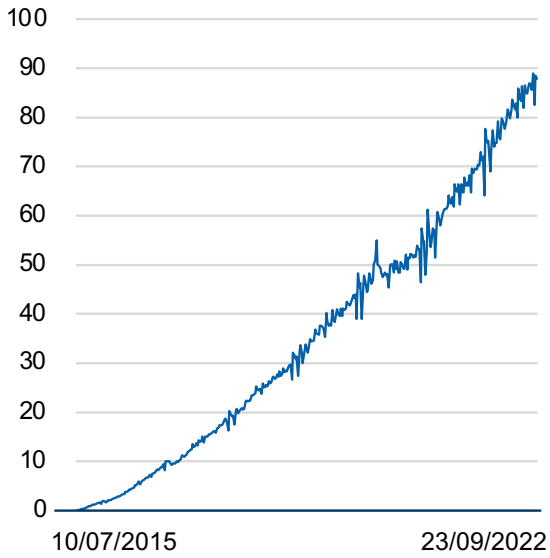
Sales evolution

USD m, % cc



US weekly TRx[1]

Total prescriptions (000)



Strong quarter performance

- WW: Increased to >8m patients on treatment[7]
- US: Accelerated momentum, ~1.1m TRx in Q3
- Europe: Strong demand growth continues

Future growth drivers

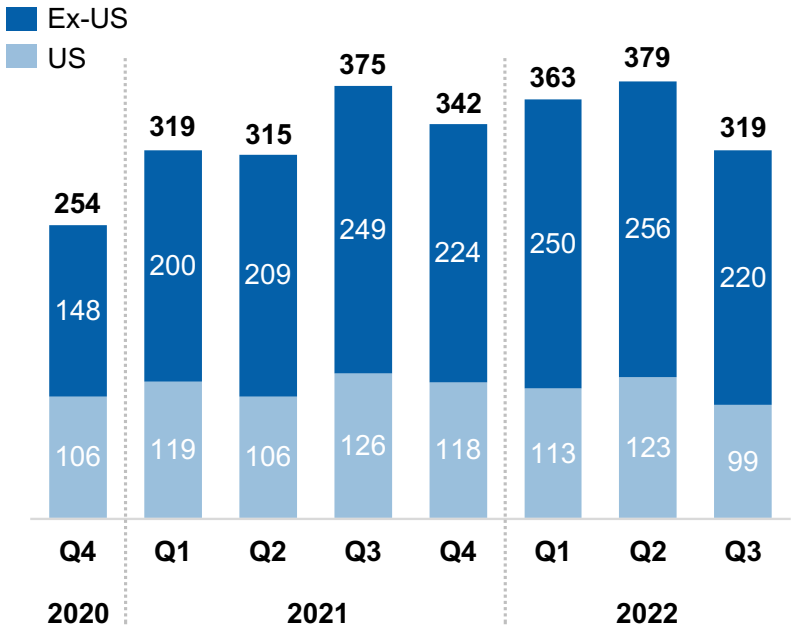
- Only 1/3 of eligible HFrEF population on treatment in G7[2]
- Strong profile in clinical and RW settings in HF[3,4]
- Guidelines (AHA/ACC/HFSA) support Entresto as 1st choice in HFrEF, expand support in HFpEF[5,6]
- Hypertension: High unmet need in Asia Pacific[6]

See last page for references TRx – Total Prescriptions WW – Worldwide HFrEF – heart failure with reduced ejection fraction RW – Real world AHA – American Heart Association ACC – American College of Cardiology HFSA – Heart Failure Society of America HFpEF – heart failure with preserved ejection fraction

Zolgensma® sales now predominantly incident patient population

Sales evolution

USD m



- Both US and ex-US market now mainly incident patient population
- YTD double digit growth in incident patients treated
- 2500+ patients treated worldwide[1]

Future growth drivers

- Foundational treatment for SMA type 1 newborns
- Now approved in 45 countries with access pathways in place in 30+
  - Access negotiations ongoing in 10+ countries (e.g. Brazil, Argentina)
- Efforts ongoing to increase newborn screening (35% in Europe; 98% in US)

IT data: STEER enrolling continues; STRENGTH to start in Q4 2022

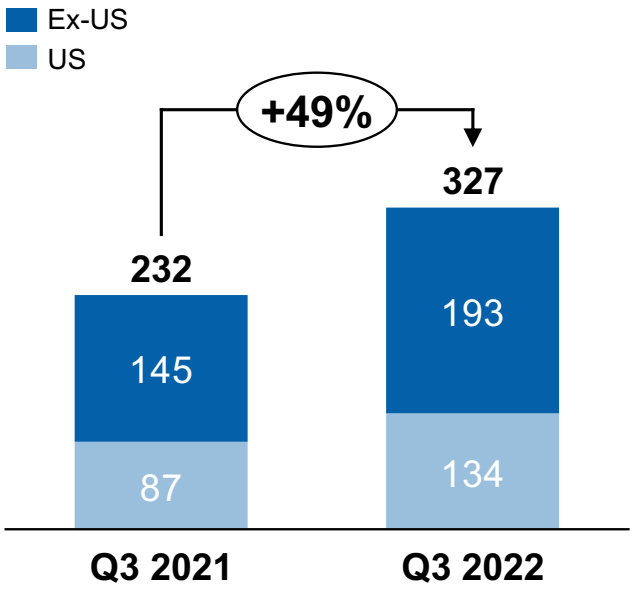
1. Across clinical trials, managed access programs and in the commercial setting.

Kisqali® grows strongly across all regions

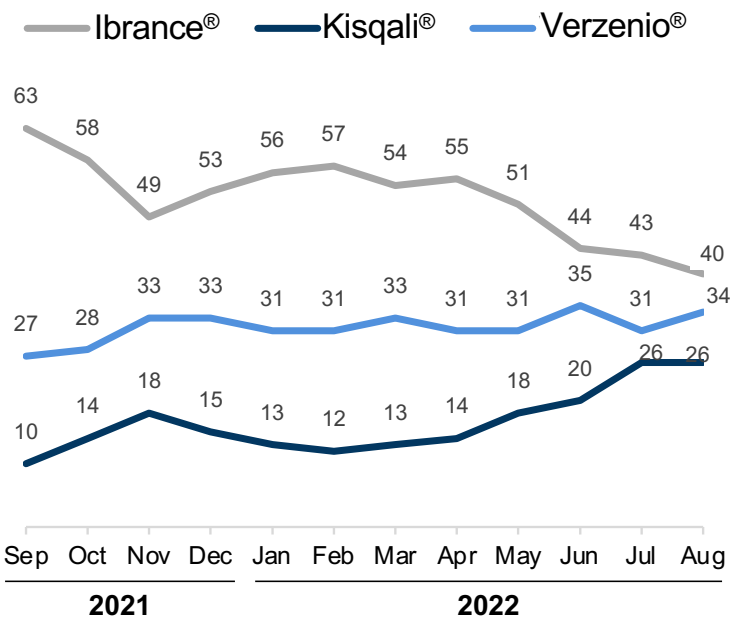
Increasing recognition of overall survival and quality of life benefits

Sales evolution

USD m, % cc



mBC monthly % NBRx share



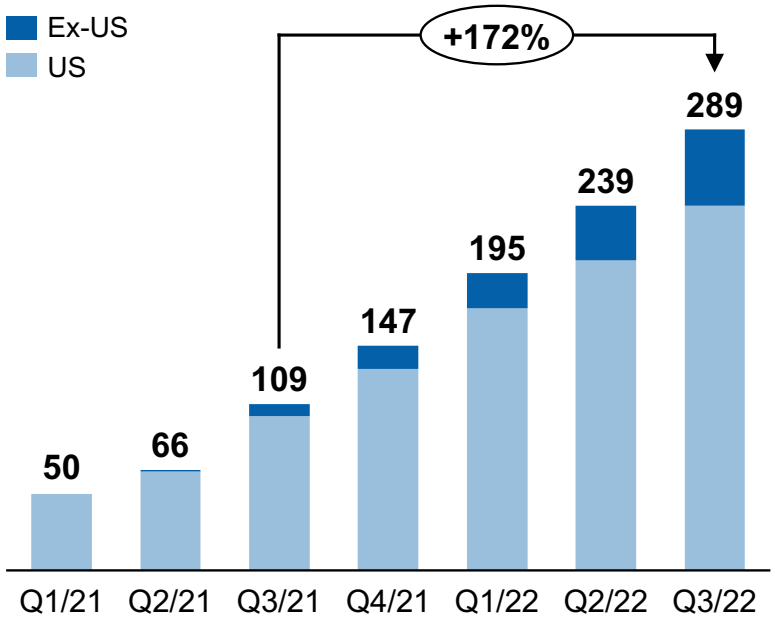
- **Sales accelerating:** US NBRx share 26% (vs. 10% PY)[1]
- Driven by **Kisqali's unique profile**:
  - Only CDK4/6 with overall survival benefit across 3 Ph3 studies
  - Improved / maintained quality of life in mBC
- **First head-to-head CDK4/6 study** (vs. Ibrance® HARMONIA): recruitment ongoing; final analysis expected 2026
- **NATALEE** adjuvant study primary analysis expected H2 2023

mBC – Metastatic breast cancer NBRx – New to Brand prescriptions CDK – Cyclin Dependent Kinase 1. Of CDK4/6 mBC market, monthly

Kesimpta® strong sales growth mainly driven by US launch momentum

Sales evolution

USD m, % cc



Launch acceleration continues[1]

- TRx +131%
- NBRx +47% vs. market -20%
- B-cell NBRx share ~30%
- Adding ~100 new writers/month
- Fast initiation within 5 days for 80% patients[2]
- >27k patients treated WW

Benefit/risk profile (new data)

- New 4-year data in recently diagnosed and treatment naive Kesimpta treated patients (subgroup) support use in early stages of RMS disease[3]

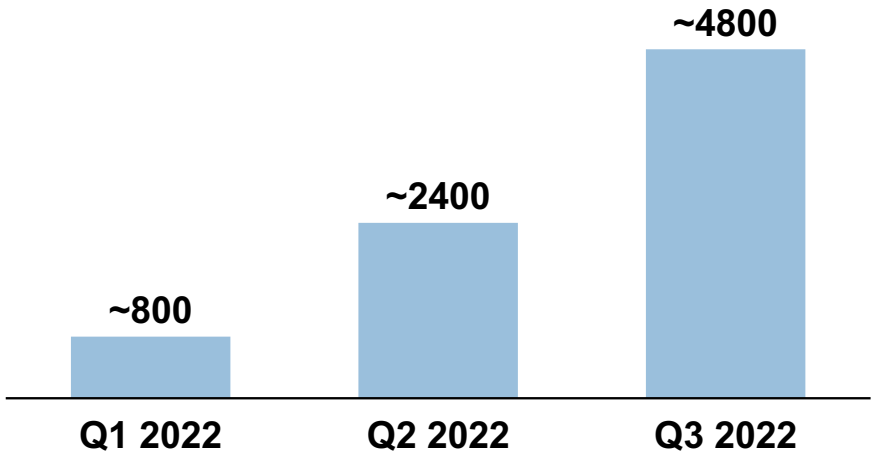
WW – worldwide TRx – Total Prescriptions NBRx – New to brand Prescription DMT – Disease Modifying Therapy 1. Refers to US unless otherwise stated 2. Time to bridge. Data on file 3. Data from ALITHIOS study. Analysis compares continuous treatment with Kesimpta and later switch from teriflunomide in recently diagnosed treatment naive patients (subgroup). Gartner J et al. Longer-term Safety and Efficacy of Ofatumumab in Recently Diagnosed and Treatment Naïve Patients is Consistent with the Overall Population in the ALITHIOS Open-Label Extension Study. Poster presented at ECTRIMS 2022. P052.

Leqvio® US launch – steadily building the foundation

Expect continued steady ramp through H1 2023

Driving broad HCP adoption

HCPs initiating a patient[1] on Leqvio®



- Q3 sales USD 34m
- HCP adoption doubled vs. Q2, focus on breadth + depth
- **Broad access:** 70% coverage at-or-near label
- **Favorable affordability:** 2/3 of patients with zero co-pay
- Free Trial Offer launched to support patient initiation[2]
- Working through **practice logistics and administration**
- AHA: 4-year efficacy and safety data to be presented (ORION-3)

HCP – Healthcare Professional AHA – American Heart Association 1. Either prescribe Leqvio® to a patient based on service center data, data on file or have ordered through Free Trial Offer program. 2. Free Trial Offer program allows HCP to order one free dose per lifetime per patient. *Leqvio® is administered initially, again at 3 months, and then once every 6 months.

Pluvicto™ continues strong start in the US

PLUVICTO™

Rapid launch uptake in US

- ☑ Q3 sales of USD 80m; NBRx share 14% in post-taxane mCRPC
- ☑ Over 120 centers actively ordering; focus in Q3 on smooth supply and customer service
- ☑ More than 75% of insured lives covered (across Medicare, Medicaid and private payers)
- ☑ Permanent A code effective in October

Preparing for further expansion

- ☑ Steadily expanding treatment centers in the US
- ☑ Significantly increasing manufacturing capacity (Ivrea in 2022; Millburn and Indianapolis planned in 2023)
- ☑ Positive CHMP opinion[2]; expected EU rollout 2023
- ☑ Earlier line studies on track:
  - PSMAfore (pre-taxane) readout expected YE 2022[1]
  - PSMAddition (mHSPC) readout expected 2024

CHMP – Committee for Human Medicinal Products NBRx – New to Brand prescriptions mCRPC – metastatic castration-resistant prostate cancer 1. Event-driven, could move to early 2023. 2. Oct 2022.

Scemblix® continues strong launch momentum in Q3

SCEMBLIX®

Strong early launch uptake

- ☑ **$41m** Q3 sales driven by patients with resistance/intolerance to other TKIs
- ☑ **13%** 3L+ total patient share[1], 2x ponatinib in 8 months
- ☑ **39%** 3L+ new patient share[1]

Future growth drivers

US Accelerated approval converted to regular approval based on 96wk data

Global Rollout ongoing with EU approval in Q3; strong early uptake in JP and UK

1L Ph3 study enrolling ahead of plan, readout expected H2 2024

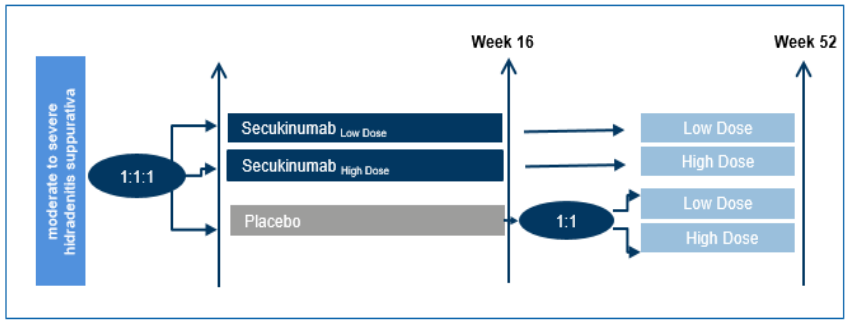
TKI – Tyrosine Kinase Inhibitor 1. IQVIA Market Sizing "Source of Business" and "Product Summary" reports as of September 2022.

Cosentyx® – rapid and sustained efficacy in Hidradenitis Suppurativa up to 52 weeks

High unmet need

~1 in 100	people affected by HS[1]
~95%	eligible patients not on biologic[2]
~50%	biologic-treated patients lose response[3]

Cosentyx® Ph3 data (SUNSHINE, SUNRISE)[4]

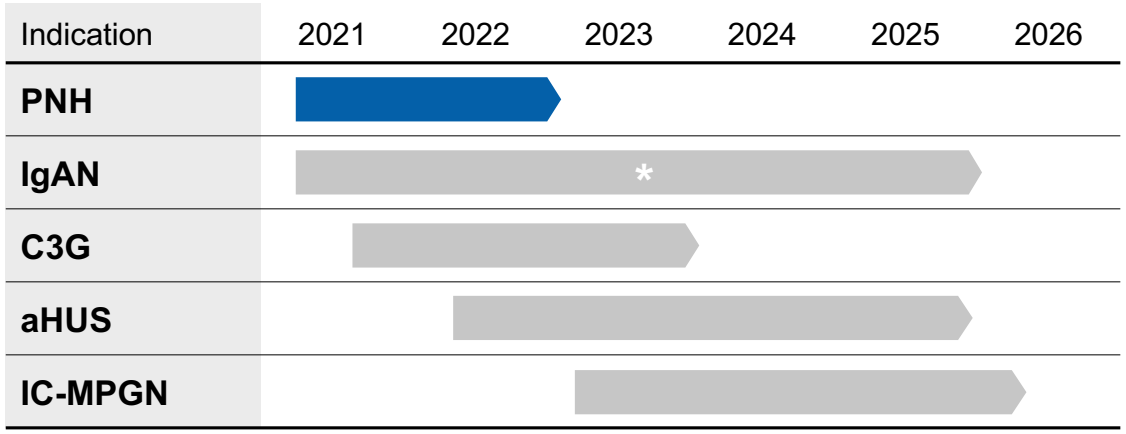


- The primary endpoint is the HiSCR at Week 16
- HiSCR response: At least a 50% decrease in abscess and Inflammatory Nodule count with no increase in the number of abscesses and/or draining tunnels

- ☑ **Rapid relief** from pain, flares, lesions, while improving quality of life
- ☑ **Sustained response** up to **52 weeks**[5]
- ☑ **Favorable safety** reinforced across 5 systemic conditions

HS – Hidradenitis Suppurativa HiSCR – Hidradenitis Suppurativa Clinical Response 1. MedLine Plus. Hidradenitis suppurativa [online] [Last accessed: Oct 2022]. 2. G6 market estimations based on IQVIA PADDS 2021.
3. Kimball A, et al. N Engl J Med. 2016;375:422–434. 4. Kimball A, et al. LB-3549 presented at EADV Congress 2022. 5. Topline results based on interim analysis where 95% of Ph3 study patients completed or discontinued by Week 52.

PNH first pivotal read-out for iptacopan "pipeline in a pill" with combined multi-blockbuster potential



Phase 3 studies initiated or planned
* 9 months readout may support US submission for accelerated approval

Multi-blockbuster potential across indications

Paroxysmal nocturnal hemoglobinuria (PNH) Phase 3 trials

APPLY-PNH

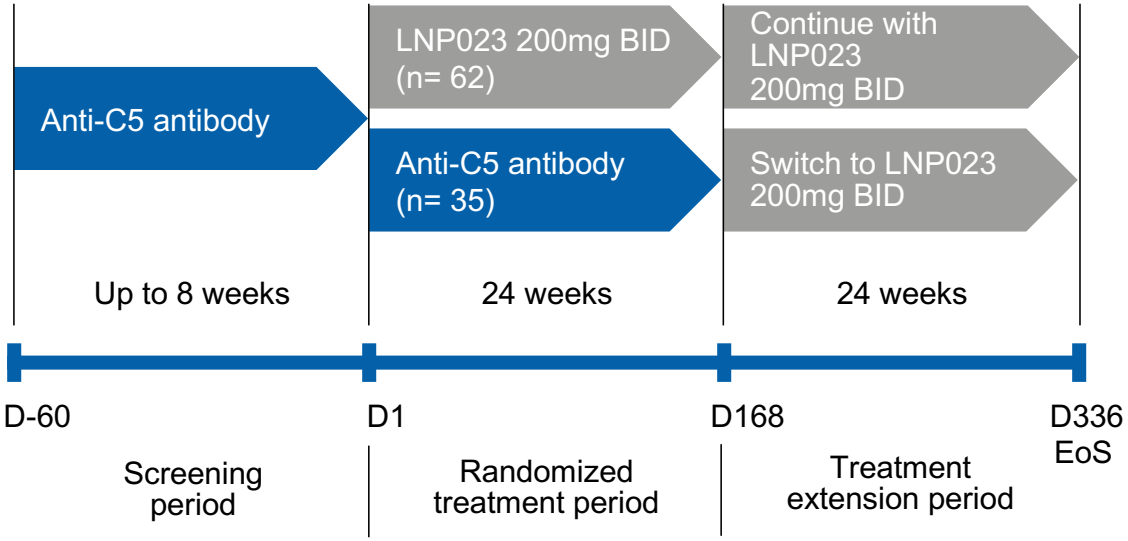
both primary endpoints of superiority vs. anti-C5 antibody met

APPOINT-PNH

in patients naive to anti-C5 antibody therapy expected to read out in 2022

IgAN – IgA nephropathy C3G – C3 glomerulopathy IC-MPGN – Immune Complex Membranoproliferative glomerulonephritis aHUS – atypical hemolytic uremic syndrome PNH – paroxysmal nocturnal hemoglobinuria

APPLY-PNH demonstrated clinically meaningful superiority vs. anti-C5



Population (n = 97)

Adult PNH patients with residual anemia (Hb <10g/dL) on a stable regimen of anti-C5 therapy 6 months prior to randomization

Primary endpoints

- ☑ Superiority for proportion of patients achieving increase in Hb ≥2g/dL from baseline in the absence of RBC transfusion
- ☑ Superiority for proportion of patients achieving Hb ≥12g/dL in the absence of RBC transfusion

Oral monotherapy iptacopan demonstrates clinically meaningful superiority over anti-C5 treatment in Ph3
Met 2 primary endpoints for superiority in PNH patients with residual anemia despite prior anti-C5 treatment

PNH – paroxysmal nocturnal hemoglobinuria Hb – Hemoglobin RBC – Red Blood Cell BID – twice a day EoS – end of study

Iptacopan has the potential to be a first line, oral complement inhibitor mono-therapy in patients with PNH

Unmet need in PNH[1-5]

10-20 cases/million; US 4-6k

~40% **remain anemic** (Hb <10g/dl) **despite** anti-C5 treatments (eculizumab / ravulizumab)

~50% of these receive transfusions

Iptacopan PNH value proposition

- ☑ Addresses both **intra- and extravascular hemolysis**, resulting in improvement of Hb levels
  - **Potential for lower transfusion requirements**
  - Potential for improved quality of life
- ☑ Potentially first **oral** administration, offering significant convenience to patients
- ☑ Potential for broad **first line label**

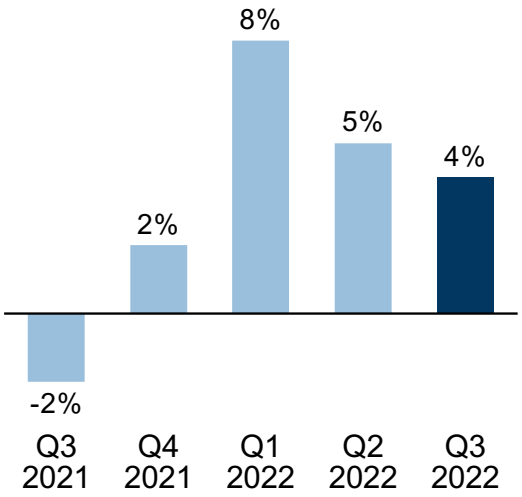
PNH – Paroxysmal nocturnal hemoglobinuria Hb – Hemoglobin 1. Cançado RD, 2021. 2. Jalbert JJ, 2019. 3. Mon Pere N, 2018. 4. Debureaux PE 2021 5. Petropoulou AD 2010.

Sandoz delivers another quarter of growth

Driven by Biopharma and ex-US sales

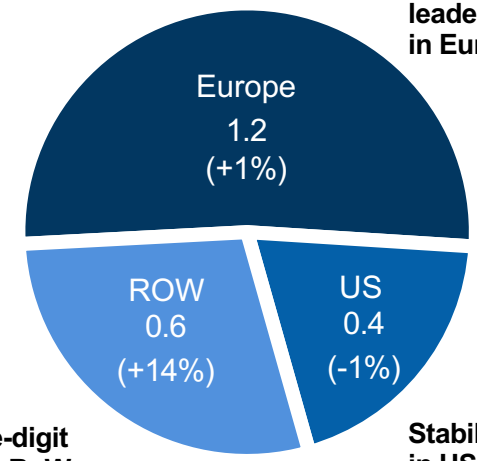
Growth rates

In cc



Q3 sales

% cc, USD bn



Maintaining leadership in Europe

Double-digit growth RoW

Stabilizing in US

Sales growth driven by biopharmaceuticals

Solid top line growth; 4th consecutive quarter

- Russia/Ukraine impact offset by strong growth in rest of Europe
- Absorbing inflation, M&S investments tied to sales growth

2022 FY guidance revised upwards

- Sales: Grow low to mid SD (from low SD)
- Core OpInc: Grow low SD (from broadly in line)

Biosimilars main future growth driver

- Targeting USD 80bn originator sales (2030) with strong pipeline of 15+ biosimilar assets
- FDA file acceptance for adalimumab HCF and natalizumab
- Positive Ph3 results for denosumab

Selectively pursuing small molecule opportunities

Novartis concluded that separation of Sandoz, via 100% spin-off, is in the best interests of shareholders; completion planned for H2 2023

HCF – High concentration formulation

Harry Kirsch

Chief Financial Officer

Financial review and 2022 guidance

Solid Q3 and YTD performance

Group[1] USD million	Q3 2022	Change vs. PY % USD	Change vs. PY % cc	9M 2022	Change vs. PY % USD	Change vs. PY % cc
Net Sales	12,543	-4	4	37,855	-1	5
Core Operating income	4,282	-4	5	12,635	-1	6
Operating income	2,168	-33	-23	7,248	-21	-13
Net Income	1,575	-43	-33	5,489	-29	-20
Growth ex. prior year Roche income		*-38*	*-27*		*-21*	*-12*
Core EPS (USD)	1.58	-8	1	4.60	-6	2
Growth ex. prior year Roche income		*1*	*10*		*3*	*11*
EPS (USD)	0.73	-41	-31	2.50	-27	-19
Growth ex. prior year Roche income		*-35*	*-25*		*-20*	*-10*
Free Cash Flow	4,169	-6		8,393	-18	
Growth ex. prior year Roche dividend		*-6*			*-14*	

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 49 of the Condensed Financial Report. A table showing the Q3 2022 and 9M 2022 key figures excluding Roche can be found on page 9 and a reconciliation of 2021 IFRS results and non-IFRS measures core results to exclude the impacts of the 2021 divestment of our Roche investment can be found on page 57 of the Condensed Interim Financial Report.

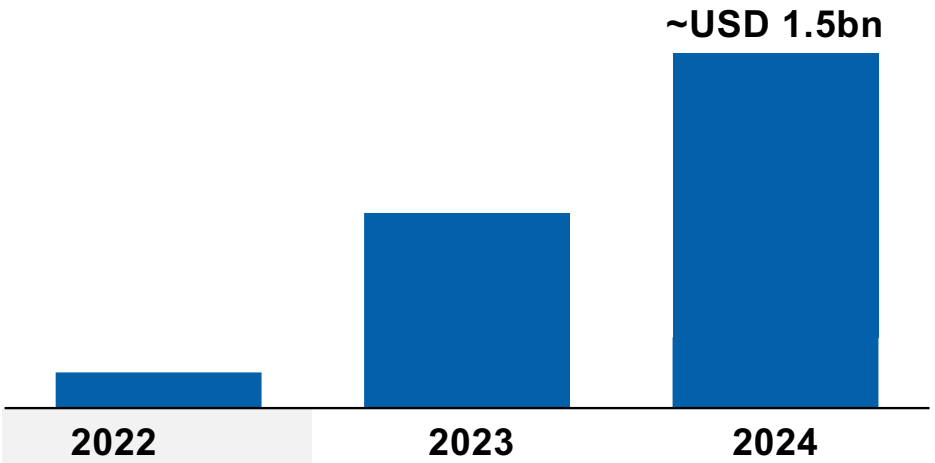
Continuing core margin improvements for Group driven by IM

	Q3 2022				9M 2022			
	Net sales change vs. PY (in % cc)[1]	Core operating income change vs. PY (in % cc)[1]	Core margin[1] (%)	Core margin change vs. PY (%pts cc)[1]	Net sales change vs. PY (in % cc)[1]	Core operating income change vs. PY (in % cc)[1]	Core margin[1] (%)	Core margin change vs. PY (%pts cc)[1]
Innovative Medicines	4	7	38.1	1.0	5	6	37.1	0.5
Sandoz	4	-5	22.3	-2.2	6	5	21.9	-0.2
Group	4	5	34.1	0.2	5	6	33.4	0.5

IM – Innovative Medicines 1. Constant currencies (cc), core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 49 of the Condensed Interim Financial Report.

On track to deliver operational efficiencies

Savings of ~USD 1.5bn to be fully embedded by 2024



Minimal impact, offsetting energy costs and inflation pressure in supply chain

Integrated Operations unit synergies

Simplification of M&S structure (non-customer-facing)

Streamlining G&A functions

One-time restructuring cost estimated at 1 to 1.2x annual structural savings

2022 full year guidance

Expected, barring unforeseen events; growth vs. PY in cc

Innovative Medicines	Sales to **grow mid single digit** Core OpInc to **grow mid to high single digit, ahead of sales**
Sandoz	Sales to **grow low to mid single digit** (**revised upwards** from to grow low single digit) Core OpInc to **grow low single digit** (**revised upwards** from broadly in line)
Group	**Sales to grow mid single digit** **Core OpInc to grow mid single digit**

Key assumptions

- Our guidance assumes that we see a continuing return to normal global healthcare systems, including prescription dynamics, and that no Sandostatin® LAR generics enter in the US.
- In June 2022, an appeals court held the Gilenya US dosing regimen patent invalid. Novartis will file a petition seeking further review with the US Supreme Court, which denied a motion to stay the issuance of the formal appeal mandate while further review is ongoing. FDA-approved Gilenya generics now launched in the US. In Q3, Gilenya US sales were USD 326 million.

FY 2022 guidance on other financial KPIs

Barring unforeseen events; growth vs. PY in cc

Group | full year guidance

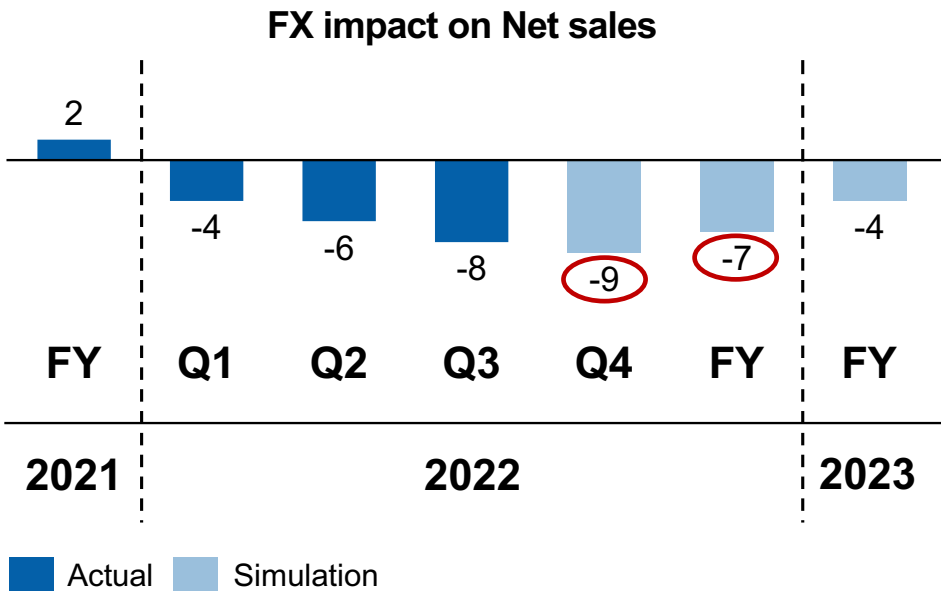
vs. PY (cc)

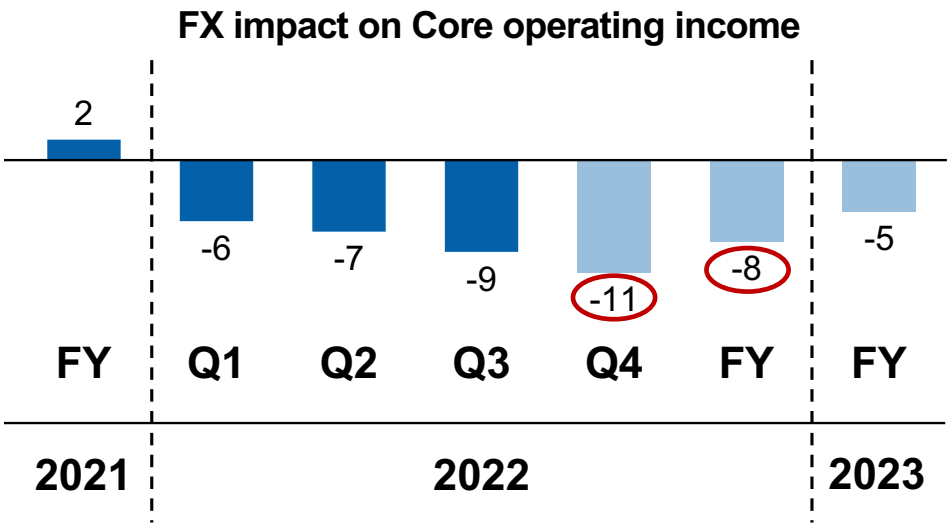
Core Net Financial Result	Expenses expected to decrease by around 100-150m vs. 2021 (revised from broadly in line vs. 2021)
Core Tax Rate	Core tax rate expected to be around 16.5% (revised from 17-17.5%)

Expected currency impact for full year 2022 and 2023

Currency impact vs. PY

%pts, assuming late October exchange rates prevail in 2022 and 2023





Vas Narasimhan

Chief Executive Officer

New Novartis: Our strategy

Deliver high-value medicines that alleviate society's greatest disease burdens through technology leadership in R&D and novel access approaches

Our focus

5 core Therapeutic Areas[1]

Cardiovascular, Immunology, Neuroscience, Solid Tumors, Hematology

2 + 3 technology platforms

Chemistry, Biotherapeutics

xRNA, Radioligand, Gene & Cell Therapy

4 priority geographies

US, China, Germany, Japan

Our priorities

Accelerate growth

Deliver **high-value medicines** (including launch excellence)

Deliver returns

Embed **operational excellence**

Strengthen foundations

Unleash the power of our **people**

Scale **data science and technology**

Build trust with **society**

1. Other TAs opportunistically.

Novartis maintains growth momentum, confirms FY 2022 guidance

Top 2022 priorities on track

1 **Successful launches:** Leqvio (laying the foundation), Kesimpta, Pluvicto, Scemblix

2 **Maintain growth momentum:** 

3 **Progress pipeline**: Multiple assets with significant sales potential, approval by 2026, on track

4 **New focused strategy:** Spin Sandoz[1]; "pure-play" IM; 5 core TAs[2]; 2+3 technology platforms[3]

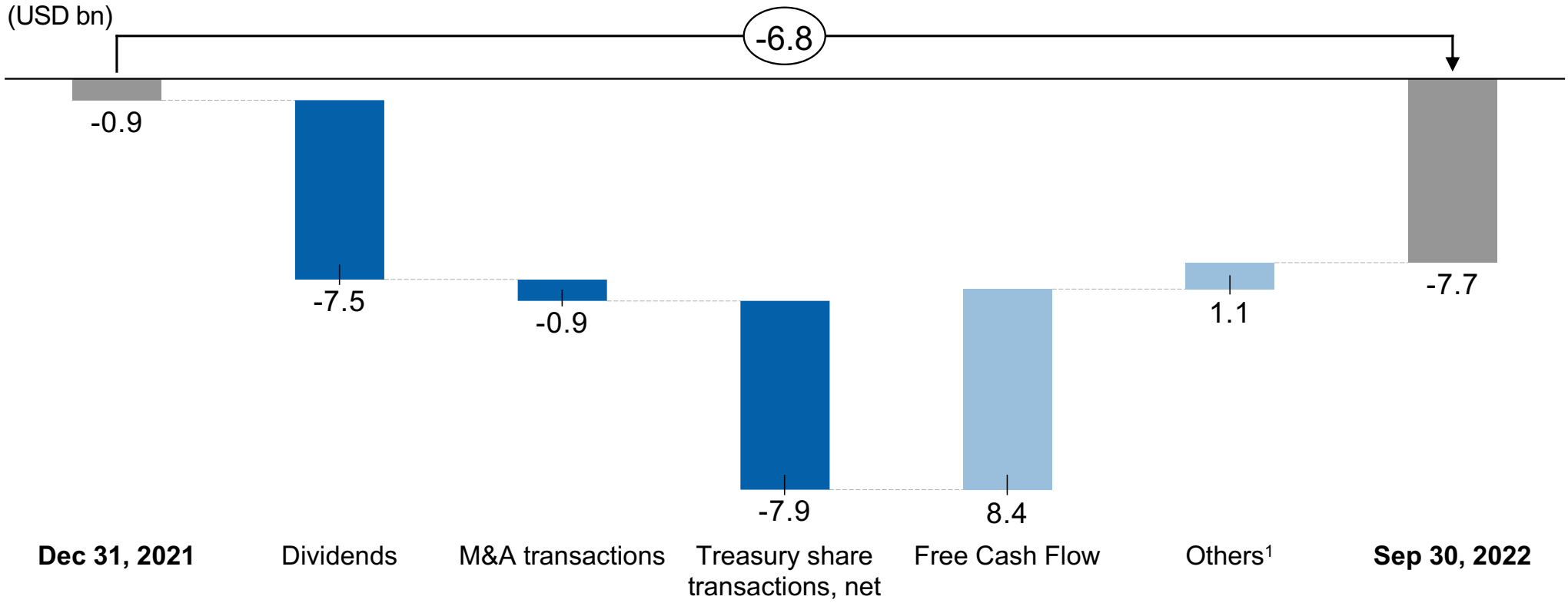
5 **Deliver returns:** Continue productivity initiatives. New organizational model being implemented

6 **Strengthen foundations**: Culture to drive performance, data science to drive value, ESG leadership

IM – Innovative Medicines TAs – Therapeutic areas 1. Intention to separate Sandoz via 100% spin-off; completion planned H2 2023. 2. Cardiovascular, immunology, neuroscience, solid tumors, hematology. 3. Two established (chemistry and biotherapeutics), three emerging (gene & cell therapy, radioligand therapy, and xRNA).

Appendix

Net debt increased by USD 6.8bn mainly due to dividends and share buybacks, partially offset by FCF



1. Key elements: Currency impact on financial debt and derivative liabilities USD 1.4bn; Payments of lease liabilities USD -0.2bn.

Broad pipeline of novel medicines continued to evolve in Q3

Core therapeutic areas

Cardiovascular | Immunology | Neuroscience | Solid tumors | Hematology

Approvals

Scemblix®	EU: CML 3L
Kymriah®	JP: r/r follicular lymphoma
Cosentyx®	JP: 300mg auto-injector

Readouts and publications

Iptacopan	Ph3 – PNH
Canakinumab	Ph3 – Adjuvant NSCLC *(PE not met)*
Tislelizumab	Ph3 – 1L HCC *(BeiGene study)*
UNR844	Ph2 – Presbyopia *(PE not met)*

Designations and milestones

Pluvicto®	mCRPC CHMP positive opinion *(post Q3)* Incl. for Locametz (imaging agent)
Ianalumab	Ph3 – Lupus Nephritis SIRIUS-LN initiated
Ianalumab	Ph3 – Sjögren's NEPTUNUS-1 and -2 initiated

Submissions

Cosentyx®	US: Hidradenitis suppurativa
Tafinlar® + Mekinist®	US: Pediatric Low-Grade Glioma (granted FDA priority review)
Xolair®	US and EU: Auto-injector

Selected milestones PE: Primary Endpoint

2022 events[1] (expected)

NME Lead

✓ **Achieved to plan** ✕ **Not achieved to plan**

Regulatory decisions	H1	Pluvicto™ mCRPC (US ✓ /EU)
	H1	Vijoice® PROS (US ✓)
	H2	Scemblix® 3L CML (JP ✓ /EU ✓)
	H2	tislelizumab ESCC 2L (US)[10]
	H1/H2	Jakavi® acute & chronic GVHD (EU ✓ /JP)
	H1/H2	Kymriah® r/r follicular lymphoma (US ✓ /EU ✓ /JP ✓)
	H1/H2	Beovu® DME (US ✓ /EU ✓ /JP ✓)
Submissions	H1	ensovibep COVID-19 (US ✓)
	H1/H2	Cosentyx® HS (EU ✓ /US ✓)
	H1/H2	tislelizumab NSCLC (EU ✓ /US x[2])
	H2	tislelizumab 1L Nasopharyngeal cancer (US x[2])
	H2	Cosentyx® Psoriatic Arthritis IV (US)
Submissions-enabling readouts	H2	canakinumab NSCLC Ph3 CANOPY-A ✓ *(PE not met)*
	H2	iptacopan PNH Ph3 APPLY-PNH ✓
	H2	Pluvicto™ pre-taxane mCRPC Ph3 PSMAfore[3]

Other readouts	H1	sabatolimab HR-MDS Ph2 ✓[4]
	H1	Cosentyx® Lichen planus Ph2 PRELUDE[5] ✓ *(PE not met)*
	H1	Cosentyx® axSpA IV Ph3 INVIGORATE-1 ✓
	H1	icenticaftor COPD Ph2b ✓[6]
	H2	UNR844 presbyopia Ph2 READER ✓ *(PE not met)*
Ph3/pivotal study starts	H1	Cosentyx® peripheral SpA x[7]
	H1	OAV101 SMA IT STEER ✓
	H1	ensovibep COVID-19 (EMPATHY Part B) x[8]
	H2	JDQ443 NSCLC mono ✓
	H2	ianalumab Sjögren's Syndrome ✓
	H2	ianalumab Lupus Nephritis ✓
	H2	ociperlimab solid tumors
	H2	Pluvicto™ nmCRPC x[11]
	H2	YTB323 2L DLBCL[9]
	H2	OAV101 SMA IT Ph3b STRENGTH

PE: Primary Endpoint Note: Kisqali® NATALEE Ph3 readout removed (2023 event as shared at Q1 2023) 1. Selected. 2. No US submission planned. 3. Could move to early 2023. 4. Submission will be based on Ph3 results. 5. Primary endpoint at Wk16 not met. 6. Ph2b DRF demonstrated dose response across multiple endpoints, study results presentation end 2022. Out-licensing planned. 7. Strategy update. 8. No definite start date for the IV Ph3 clinical trial can be provided at this time. 9. Development strategy being updated. 10. FDA deferred action pending completion of required inspections. 11. Ph3 in nmCRPC shifting to Ph2, with FPFV in 2023.

Confident in future growth driven by our strength and depth in cardiovascular, immunology, neuroscience...

Selected assets, nearly all with exclusivity into 2030+

Cardiovascular

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Leqvio®	CVRR-LDLC	●●●	Ph3 ORION-4 and VICTORION-2-PREVENT ongoing	2026+
			Primary prevention initiation	-
Iptacopan[1]	IgAN	●●●	Ph3 APPLAUSE-IgAN ongoing	2023[2]
	C3G		Ph3 APPEAR-C3G ongoing	2023
	iMN		Ph2b ongoing	2026+
Pelacarsen	CVRR-Lp(a)	●●●	Ph3 Lp(a)HORIZON ongoing	2025

Neuroscience

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Zolgensma®	SMA IT	●●●	Ph3 STEER ongoing	2025
Branaplam	Huntington's disease	●●●	Ph2b VIBRANT-HD ongoing	2026+
Remibrutinib[1]	Multiple sclerosis	●●●	Ph3 REMODEL-1 and -2 ongoing	2026+
DLX313 (UCB0599)	Parkinson's disease	●●●	Ph2 ongoing	2026+

Immunology

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Cosentyx®	HS		Submitted in US	
	GCA	●●●	Ph3 GCAPTAIN ongoing	2025
	Lupus Nephritis		Ph3 SELUNE ongoing	2026+
Ligelizumab	Food allergy	●●●	Ph3 ongoing	2025
Remibrutinib[1]	CSU	●●●	Ph3 REMIX-1 and -2 ongoing	2024
	Other indications being explored			
Ianalumab	Sjögren's	●●●	Ph3 NEPTUNUS-1 and -2 started	2026+
	SLE		Ph2a ongoing	2026+
	Autoimmune hepatitis		Ph2b AMBER ongoing	2026+
	Lupus Nephritis		Ph3 SIRIUS-LN started	2026+
Iscalimab	Sjögren's	●●	Ph2b TWINSS ongoing	2026+
	HS		Ph2a ongoing	2026+

Unprobabilized peak sales (USD) : ● <1bn ●● 1-2bn ●●● >2bn

'Bold Bets' | LNA043 (osteoarthritis: Ph2b ONWARDS ongoing), SAF312 (COSP: Ph2b ongoing)

1. Peak sales potential based on all studied indications. 2. Based on 9 months UPCR readout (US accelerated approval)

… and strength and depth in oncology

Selected assets, nearly all with exclusivity into 2030+

Solid Tumors

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Kisqali®	HR+/HER2- BC (adj)	●●●	Ph3 NATALEE readout event-driven, expected 2023	2023
Canakinumab	NSCLC adjuvant	●●	Ph3 CANOPY-A did not meet primary endpoint	
Pluvicto™	mCRPC post-taxane	●●●	US approved	-
	mCRPC pre-taxane		Ph3 PSMAfore readout event-driven, end 2022[1]	2023
	mHSPC		Ph3 PSMAddition ongoing	2024
JDQ443 KRAS inhibitor	2/3L NSCLC (mono)	●●●	Ph3 ongoing	2024
	NSCLC (combo)		Ph2 ongoing	2026+
TNO155 SHP2 inhibitor	Solid tumors: multiple combinations being explored in ongoing trials			
Tislelizumab[2]	2L esophageal cancer	●●	Submitted in EU	-
	NSCLC		Submitted in EU	-
	Other indications		Ongoing trials	-
Ociperlimab[2] TIGIT mab	NSCLC	●●●	Ph3 ongoing[3]	
	Other indications		Ongoing trials[3]; additional Ph3 study initiation H2 2022	

Hematology

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Scemblix® (asciminib)	CML 3L	●●●	EU Approval	-
	CML 1L		Ph3 ongoing	2025
Iptacopan[2]	PNH	●●●	Primary endpoint met (Ph3 APPLY-PNH); readout in 2022 (Ph3 APPOINT-PNH)	2023
	aHUS		Ph3 ongoing	2025
Sabatolimab	HR-MDS	●●●	Ph3 STIMULUS-MDS-2 ongoing	2024
	AML		Ph2 STIMULUS-AML-1 ongoing	2026+
YTB323 CD19 CAR-T	Non-Hodgkin's Lymphoma	●●●	Plans under review	2025[4]
PHE885 BCMA CART-T	Multiple myeloma	●	Ph2 ongoing	2025

Unprobabilized peak sales (USD): ● <1bn ●● 1-2bn ●●● >2bn

'Bold Bets' | NIS793 (1L mPDAC: Ph3 ongoing, 1L metastatic colorectal cancer: Ph2 ongoing)

1. Could move to early 2023. 2. Peak sales potential based on all studied indications; Novartis territories. 3. Active trials are being conducted by BeiGene, option deal. 4. Development strategy being updated.

Our pipeline projects at a glance

	Phase 1/2	Phase 3	Registration	Total
Innovative medicines	**97**	**44**	**6**	**147**
Solid Tumors	23	17	3	43
Hematology	19	6	0	25
Immunology	22	9	2	33
Neuroscience	6	5	0	11
Cardiovascular	8	5	1	14
Others	19	2	0	21
Ophthalmology	*5*	*1*	*0*	*6*
Respiratory & Allergy	*4*	*0*	*0*	*4*
Global Health	*10*	*1*	*0*	*11*
Biosimilars[1]	**0**	**2**	**0**	**2**
Total	**97**	**46**	**6**	**149**

1. Selected disclosed, internal projects.

Novartis pipeline in Phase 1

30 lead indications

Lead indication

Solid tumors

Code	Name	Mechanism	Indication(s)
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Multiple solid tumors
AAA817	Ac-PSMA-617	Radioligand therapy target PSMA	Metastatic castration-resistant prostate cancer
ADPT01	ADPT01	-	Colorectal cancer (combos)
DFF332	DFF332	HIF2A inhibitor	Renal cell carcinoma
DKY709	DKY709 + spartalizumab	Novel immunomodulatory agent	Cancers
DYP688	DYP688	GNAQ,GNA11 antagonist	Unveal melanoma
IAG933	IAG933	-	Mesothelioma
JDQ443	JDQ443	KRAS inhibitor	KRAS G12C mutated solid tumors
KAZ954	KAZ954	-	Solid tumors
MGY825	MGY825	-	NSCLC
NIS793	NIS793, spartalizumab	TGFB inhibitor	Solid tumors
NIZ985	NIZ985, spartalizumab	IL-15 agonist	Solid tumors
NZV930	NZV930, spartalizumab, NIR178	CD73 antagonist	Solid tumors
TNO155	TNO155	SHP2 inhibitor	Solid tumors (combo)
VPM087	gevokizumab	IL-1 beta antagonist	Colorectal cancer, 1st line
WNT974	WNT974 + spartalizumab	Porcupine inhibitor	Solid tumors

Immunology

Code	Name	Mechanism	Indication(s)
FIA586	FIA586	-	Non-alcoholic steatohepatitis (NASH)
MHS552	MHS552	-	Autoimmune indications
MHV370	MHV370	-	Systemic lupus erythematosus
NGI226	NGI226	-	Tendinopathy

Neuroscience

Code	Name	Mechanism	Indication(s)
NIO752	NIO752	Tau antagonist	Progressive supranuclear palsy

Hematology

Code	Name	Mechanism	Indication(s)
ADPT03	ADPT03	BCL11A	Sickle cell anemia
HDM201	HDM201 (combos)	MDM2 inhibitor	Haematological malignancy
JBH492	JBH492	-	Haematological malignancy
JEZ567	JEZ567	CD123 CAR-T	Acute myeloid leukaemia
MAK683	MAK683	EED inhibitor	Cancers
MBG453	sabatolimab	TIM3 antagonist	Low risk myelodysplastic syndrome
MIK665	MIK665	MCL1 inhibitor	Hematological malignancies
VAY736	ianalumab + ibrutinib	BAFF-R inhibitor	Haematological malignancy (combo)
VOB560	VOB560	-	Cancers
WVT078	WVT078	-	Multiple myeloma
YTB323	YTB323	CD19 CAR-T	DLBCL and adult ALL

Cardiovascular

Code	Name	Mechanism	Indication(s)
XXB750	XXB750	-	Cardiovascular diseases

Others

Code	Name	Mechanism	Indication(s)
Global Health			
EDI048	EDI048	CpPI(4)K inhibitor	Cryptosporidiosis
EYU688	EYU688	NS4B inhibitor	Dengue
KAF156	ganaplacide	-	Malaria prophylaxis
INE963	INE963	-	Malaria, uncomplicated
Respiratory & Allergy			
NCJ424	NCJ424	-	Respiratory diseases
Ophthalmology			
MHU650	MHU650	-	Diabetic eye diseases

Novartis pipeline in Phase 2

27 lead indications

Lead indication

Solid Tumors

Code	Name	Mechanism	Indication(s)
AAA601	Lutathera®	Radioligand therapy target SSTR	GEPNET, pediatrics
			1L ES-SCLC
			Glioblastoma
JDQ443	JDQ443	KRAS inhibitor	NSCLC (combo)
NIR178	NIR178, spartalizumab	Ad2AR inhibitor, PD1 inhibitor	Cancers
NIS793	NIS793	TGFB inhibitor	1L metastatic colorectal cancer
TNO155	TNO155	SHP2 inhibitor	Solid tumors (single agent)

Immunology

Code	Name	Mechanism	Indication(s)	
CFZ533	iscalimab	CD40 inhibitor	Sjögren's	Hidradenitis suppurativa
CMK389	CMK389	IL-18 inhibitor	Atopic dermatitis	
DFV890	DFV890	NLRP3 inhibitor	Osteoarthritis	
			Familial cold auto-inflammatory syndrome	
LNA043	LNA043	ANGPTL3 agonist	Knee osteoarthritis	
			Osteoarthritis (combos)	
LOU064	remibrutinib	BTK inhibitor	Food allergy	
			Hidradenitis suppurativa	
			Sjögren's	
LRX712	LRX712	-	Osteoarthritis	
LYS006	LYS006	Anti-inflammatory	Colitis ulcerative	
MAS825	MAS825	-	NLRC4-GOF indications	Hidradenitis suppurativa
MHV370	MHV370	-	Sjögren's	Mixed connective tissue disease
VAY736	ianalumab	BAFF-R inhibitor	Autoimmune hepatitis	
			Systemic lupus erythematosus	

Neuroscience

Code	Name	Mechanism	Indication(s)
ADPT06	ADPT06	-	Cognitive impairment
BLZ945	sotuletinib	CSF-1R inhibitor	Amyotrophic lateral sclerosis
DLX313	DLX313 (UCB0599)	Alpha-synuclein Inhibitor	Parkinson's disease
LMI070	branaplam	mRNA splicing modulator	Huntington's disease[2]
MIJ821	MIJ821	NR2B negative allosteric modulator	Major depressive disorder with acute suicidal ideation or behavior

1. Gyroscope acquisition. 2. Plan update ongoing.

Hematology

Code	Name	Mechanism	Indication(s)	
ABL001	Scemblix®	BCR-ABL inhibitor	Chronic myeloid leukemia, 2L, pediatrics	
INC424	Jakavi®	JAK1/2 inhibitor	Acute GVHD, pediatrics	Chronic GVHD, pediatrics
LNP023	iptacopan	CFB inhibitor	Immune thrombocytopenia	
MBG453	sabatolimab	TIM3 antagonist	Unfit acute myeloid leukaemia	
			Acute myeloid leukaemia, maintenance	
PHE885	PHE885	BCMA cell therapy	4L multiple myeloma	
PKC412	Rydapt®	Multi-targeted kinase inhibitor	Acute myeloid leukemia, pediatrics	

Cardiovascular

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	
			Lupus nephritis	
MBL949	MBL949	-	Obesity related diseases	
TIN816	TIN816	ATP modulator	Acute kidney injury	

Others

Code	Name	Mechanism	Indication(s)	
Global Health				
KAE609	cipargamin	PfATP4 inhibitor	Malaria, severe	Malaria, uncomplicated
KAF156	ganaplacide	-	Malaria, uncomplicated	
LXE408	LXE408	Proteasome inhibitor	Visceral leishmaniasis	
SEG101	Adakveo®	P-selectin inhibitor	Sickle cell disease, pediatrics	
SKO136	ensovibep	Multi-specific DARPin	Corona virus infection	
Respiratory & Allergy				
CMK389	CMK389	IL-18 inhibitor	Pulmonary sarcoidosis	
LTP001	LTP001	SMURF1 inhibitor	PAH	
QMF149	Atectura®	Combo	Asthma, pediatrics	
Ophthalmology				
LKA651	LKA651	EPO inhibitor	Diabetic retinopathy	
LNP023	iptacopan	CFB inhibitor	iAMD	
PPY988[1]	PPY988	Gene therapy	Geographic atrophy	
SAF312	Libvatrep	TRPV1 antagonist	Chronic ocular surface pain	

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Novartis pipeline in Phase 3

9 lead indications

Lead indication

Solid Tumors

Code	Name	Mechanism	Indication(s)		
AAA617	Pluvicto®	Radioligand therapy target PSMA	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)		
BYL719	Piqray®	PI3Kα inhibitor	HER2+ adv BC	Triple negative breast cancer	Ovarian cancer
JDQ443	JDQ443	KRAS inhibitor	2/3L Non-small cell lung cancer		
LEE011	Kisqali®	CDK4/6 Inhibitor	HR+/HER2- BC (adj)		
NIS793	NIS793	TGFB1 inhibitor	1L Metastatic pancreatic ductal adenocarcinoma		
VDT482	Tislelizumab	PD1 inhibitor	1L Nasopharyngeal Carcinoma	Adj/Neo adj. NSCLC	
			1L ESCC	1L Gastric cancer	
			1L Hepatocellular Carcinoma	Localized ESCC	
			1L Urothelial Cell Carcinoma	1L Small Cell Lung Cancer	

Immunology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Lupus Nephritis
			Psoriatic arthritis (IV formulation)
			Axial SpA (IV formulation)
			Giant cell arteritis
IGE025	Xolair®	IgE inhibitor	Food allergy
LOU064	remibrutinib	BTK inhibitor	Chronic spontaneous urticaria
QGE031	ligelizumab	IgE inhibitor	Food allergy
VAY736	ianalumab	BAFF-R inhibitor	Sjögren's
			Lupus Nephritis

Neuroscience

Code	Name	Mechanism	Indication(s)
AMG334	Aimovig®	CGRPR antagonist	Migraine, pediatrics
BAF312	Mayzent®	S1P1,5 receptor modulator	Multiple sclerosis, pediatrics
LOU064	remibrutinib	BTK inhibitor	Multiple sclerosis
OAV101	AVXS-101	SMN1 gene replacement therapy	SMA IT administration
OMB157	Kesimpta®	CD20 Antagonist	Multiple sclerosis, pediatrics

Hematology

Code	Name	Mechanism	Indication(s)
ABL001	Scemblix®	BCR-ABL inhibitor	Chronic myeloid leukemia, 1st line
ETB115	Promacta®	Thrombopoietin receptor (TPO-R) agonist	Radiation sickness syndrome
LNP023	iptacopan	CFB inhibitor	Paroxysmal nocturnal haemoglobinuria
			Atypical haemolytic uraemic syndrome
MBG453	sabatolimab	TIM3 antagonist	Myelodysplastic syndrome
YTB323	YTB323	CD19 CAR-T	2L Diffuse large B-cell lymphoma[2)]

Cardiovascular

Code	Name	Mechanism	Indication(s)	
KJX839	Leqvio®	siRNA (regulation of LDL-C)	CVRR-LDLC	Hyperlipidemia, pediatrics
LNP023	iptacopan	CFB inhibitor	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))	

Others

Code	Name	Mechanism	Indication(s)
Global Health			
COA566	Coartem®	-	Malaria, uncomplicated (<5kg patients)
Ophthalmology			
RTH258	Beovu®	VEGF inhibitor	Diabetic retinopathy

Biosimilars

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

1. ^{177}Lu-dotatate in US. 2. Ph3 to be initiated pending strategy update.

Novartis pipeline in registration

1 lead indication

Lead indication

Solid Tumors

Code	Name	Mechanism	Indication(s)
VDT482	Tislelizumab	PD1 inhibitor	2L ESCC
			Non-small cell lung cancer
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	HGG/LGG, pediatrics

Immunology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Hidradenitis suppurativa
IGE025	Xolair®	IgE inhibitor	Auto-injector

Cardiovascular

Code	Name	Mechanism	Indication(s)
LCZ696	Entresto®	Angiotensin receptor/neprilysin inhibitor	Congestive heart failure, pediatrics[1)]

1. Approved in US.

Novartis submission schedule

New Molecular Entities: Lead and supplementary indications

LEAD INDICATIONS

2022

- **ensovibep** — Lead
  SKO136
  COVID19

2023

- **iptacopan** — Lead
  LNP023
  PNH

2024

- **JDQ443** — Lead
  JDQ443
  2/3L NSCLC (mono)
- **sabatolimab** — Lead
  MBG453
  HR-MDS
- **remibrutinib** — Lead
  LOU064
  CSU

2025

- **ligelizumab** — Lead
  QGE031
  Food allergy
- **NIS793** — Lead
  1L Pancreatic cancer
- **pelacarsen** — Lead
  TQJ230
  CVRR-Lp(a)
- **YTB323**[1] — Lead
  2L Diffuse large B-cell lymphoma

≥2026

- **^{177}Lu-NeoB** — Lead
  AAA603
  Multiple Solid Tumors
- **branaplam**[1] — Lead
  LMI070
  Huntington's disease
- **cipargamin** — Lead
  KAE609
  Malaria severe
- **ganaplacide** — Lead
  KAF156
  Malaria uncomplicated
- **gevokizumab** — Lead
  VPM087
  1st line CRC
- **iscalimab** — Lead
  CFZ533
  Sjögren's syndrome
- **ianalumab** — Lead
  VAY736
  Sjögren's syndrome
- **libvatrep** — Lead
  SAF312
  COSP
- **LNA043** — Lead
  Knee osteoarthritis
- **LXE408** — Lead
  Visceral leishmaniasis
- **MIJ821** — Lead
  Acute depression
- **PPY988**[3] — Lead
  Geographic atrophy
- **TNO155** — Lead
  Solid tumors

NEW INDICATIONS

2022

- **tislelizumab** — LCM
  VDT482
  NSCLC

2023

- **Pluvicto** — LCM
  AAA617
  mCRPC, Pre-taxane
- **iptacopan** — LCM
  LNP023
  C3G
- **iptacopan** — LCM
  LNP023
  IgAN
- **tislelizumab** — LCM
  VDT482
  1L Gastric Cancer
- **tislelizumab** — LCM
  VDT482
  1L ESCC
- **tislelizumab** — LCM
  VDT482
  Localized ESCC
- **tislelizumab** — LCM
  VDT482
  1L Hepatocellular Carcinoma
- **tislelizumab** — LCM
  VDT482
  1L Nasopharyngeal Carcinoma

2024

- **Pluvicto** — LCM
  AAA617
  mHSPC
- **tislelizumab** — LCM
  VDT482
  1L Small Cell Lung Cancer

2025

- **Scemblix** — LCM
  ABL001
  CML 1L
- **iptacopan** — LCM
  LNP023
  aHUS

≥2026

- **cipargamin** — LCM
  KAE609
  Malaria uncomplicated
- **JDQ443** — LCM
  JDQ443
  NSCLC (combo)
- **ianalumab** — LCM
  VAY736
  AIH
- **ianalumab** — LCM
  VAY736
  Lupus Nephritis
- **ianalumab** — LCM
  VAY736
  SLE
- **iptacopan** — LCM
  LNP023
  iMN
- **sabatolimab** — LCM
  MBG453
  Unfit AML
- **Scemblix** — LCM
  ABL001
  CML, 2L, pediatrics
- **remibrutinib** — LCM
  LOU064
  Sjögren's syndrome
- **remibrutinib** — LCM
  LOU064
  Multiple sclerosis
- **tislelizumab** — LCM
  VDT482
  Adj/Neo adj NSCLC
- **tislelizumab** — LCM
  VDT482
  1L Urothelial Cell Carcinoma

1. Development strategy being updated. 2. Plan update on-going. 3. Gyroscope acquisition.

Novartis submission schedule

Supplementary indications for existing brands

EXISTING BRANDS

2022

- **Cosentyx** LCM — secukinumab, AIN457 — PsA IV
- **Cosentyx** LCM — secukinumab, AIN457 — Hidradenitis suppurativa
- **Entresto EU**[1] LCM — sacubitril/valsartan, LCZ696 — Pediatric CHF
- **Tafinlar + Mekinist** LCM — dabrafenib + trametinib, DRB436 — HGG/LGG - Pediatrics
- **Xolair** LCM — omalizumab, IGE025 — Auto-injector

2023

- **Cosentyx** LCM — secukinumab, AIN457 — axSpA IV
- **denosumab** BioS — GP2411 — Osteoporosis (same as originator)
- **Kisqali** LCM — **ribociclib, LEE011** — **HR+/HER2- BC (adj)**
- **Lutathera** LCM — ^{177}Lu-oxodotreotide[2] — GEP-NET 1L G3
- **Piqray** LCM — alpelisib, BYL719 — Ovarian cancer
- **Xolair** LCM — omalizumab, IGE025 — Food allergy

2024

- **aflibercept** BioS — SOK583 — Ophthalmology indication (as originator)
- **Adakveo** LCM — SEG101 — Sickle cell disease, pediatrics
- **Coartem** LCM — artemether + lumefantrine, COA566 — Malaria uncompl., formula for <5kg
- **Jakavi** LCM — ruxolitinib, INC424 — Pediatrics Acute GVHD
- **Jakavi** LCM — ruxolitinib, INC424 — Pediatrics Chronic GVHD

2025

- **Beovu** LCM — brolucizumab, RTH258 — Diabetic retinopathy
- **Cosentyx** LCM — secukinumab, AIN457 — GCA
- **Leqvio** LCM — KJX839 — Ped Hyoerlipidemia
- **Piqray** LCM — alpelisib, BYL719 — HER2+ adv BC
- **Promacta** LCM — eltrombopag, ETB115 — Radiation sickness syndrome
- **Zolgensma** LCM — AVXS-101 OAV101 — SMA IT

≥2026

- **Atectura** LCM — indacaterol + mometasone, QMF149 — Asthma, pediatrics
- **Aimovig** LCM — erenumab, AMG334 — Pediatric Migraine
- **Cosentyx** LCM — secukinumab, AIN457 — Lupus Nephritis
- **Kesimpta**[3] LCM — ofatumumab — Multiple sclerosis, pediatrics
- **Leqvio** LCM — KJX839 — CVRR-LDLC
- **Mayzent**[3] LCM — siponimod, BAF312 — Multiple sclerosis, pediatrics
- **Piqray** LCM — alpelisib, BYL719 — TNBC
- **Rydapt** LCM — midostaurin, PKC412 — Acute myeloid leukemia, pediatrics

1. Approved in US. 2. 177Lu-dotatate in US. 3. Kesimpta and Mayzent: pediatric study in multiple sclerosis run in conjunction (NEOS).

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

iptacopan - CFB inhibitor

NCT03955445 (CLNP023B12001B)

Indication	C3 glomerulopathy (C3G)
Phase	Phase 2
Patients	27 patients from ongoing Ph2 (sample size from Ph3 pending HA discussions Q1 2021), total patients for this study will increase
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)
Arms Intervention	Open-label LNP023 200mg bid
Target Patients	Patients with C3 glomerulopathy
Read-out Milestone(s)	2025
Publication	Wong et al 2021 Nephrology, Dialysis and Transplantation Vol. 36, Suppl. 1: eGFR trajectory

iptacopan - CFB inhibitor

NCT04154787 (CLNP023D12201)

Indication	Idiopathic membranous nephropathy (iMN)
Phase	Phase 2
Patients	72
Primary Outcome Measures	Change from baseline of UPCR derived from 24hr urine collections at Baseline and Week 24
Arms Intervention	LNP023 low dose LNP023 high dose Rituximab
Target Patients	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 Milestone(s)	2023
Publication	TBD

iptacopan - CFB inhibitor

NCT04578834 APPLAUSE-IgAN (CLNP023A2301)

Indication	IgA nephropathy
Phase	Phase 3
Patients	450
Primary Outcome Measures	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 - LNP023 200mg BID Arm 2 - Placebo BID
Target Patients	Primary IgA Nephropathy patients
Read-out Milestone(s)	2023 (primary endpoint for US initial submission, 9 months UPCR) 2025 (24 months)
Publication	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

iptacopan - CFB inhibitor

NCT04817618 APPEAR-C3G (CLNP023B12301)

Indication	C3 glomerulopathy
Phase	Phase 3
Patients	68
Primary Outcome Measures	Log-transformed ratio to baseline in UPCR (sampled from a 24 hour urine collection)
Arms Intervention	Experimental: iptacopan 200mg b.i.d. Placebo Comparator: Placebo to iptacopan 200mg b.i.d.
Target Patients	Patients with native C3G
Read-out Milestone(s)	2023
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

NCT03705234 ORION-4 (CKJX839B12301)

Indication	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH)
Phase	Phase 3
Patients	15000
Primary Outcome Measures	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	Arm 1: every 6 month treatment Inclisiran sodium 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	Patient population with mean baseline LDL-C ≥ 100mg/dL
Read-out Milestone(s)	2026
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

NCT03814187 ORION-8 (CKJX839A12305B)

Indication	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH) and Homozygous Familial Hypercholesterolemia (HoFH)
Phase	Phase 3
Patients	3275
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
Arms Intervention	Inclisiran sodium 300mg on Day 90 and every 180 days for a planned duration of 3 years
Target Patients	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy and risk equivalents (patients from ORION 3, 9, 10 & 11 studies)
Read-out Milestone(s)	2023
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

NCT04652726 ORION-16 (CKJX839C12301)

Indication	Hyperlipidemia, pediatrics
Phase	Phase 3
Patients	150
Primary Outcome Measures	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.
Target Patients	Adolescents (12 to less than 18 years) with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C)
Read-out Milestone(s)	2025
Publication	Design publication in Eur. J. Prev. Cardiol. Vol. 29, Feb. 2022; Presentation at EAS May-2022

Leqvio® - siRNA (regulation of LDL-C)

NCT04659863 ORION-13 (CKJX839C12302)

Indication	Hyperlipidemia, pediatrics
Phase	Phase 3
Patients	12
Primary Outcome Measures	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.
Target Patients	Adolescents (12 to less than 18 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDL-C)
Read-out Milestone(s)	2025
Publication	Design publication in Eur. J. Prev. Cardiol. Vol. 29, Feb. 2022; Presentation at EAS May-2022

Leqvio® - siRNA (regulation of LDL-C)

NCT05030428 VICTORION-2P (CKJX839B12302)

Indication	Secondary prevention of cardiovascular events in patients with elevated levels of LDL-C
Phase	Phase 3
Patients	15000
Primary Outcome Measures	1. Time to First Occurrence of 3P-MACE (3-Point Major Adverse Cardiovascular Events)
Arms Intervention	Arm 1: Experimental Inclisiran sodium, Subcutaneous injection Arm 2: Placebo Comparator, Placebo Subcutaneous injection
Target Patients	Participants with established cardiovascular disease (CVD)
Read-out Milestone(s)	2027
Publication	TBD

pelacarsen - ASO targeting Lp(a)

NCT04023552 Lp(a)HORIZON (CTQJ230A12301)

Indication	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein(a)
Phase	Phase 3
Patients	8323
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 Milestone(s)	2025
Publication	TBD

Immunology

Cosentyx® - IL-17A inhibitor

NCT04181762 SELUNE (CAIN457Q12301)

Indication	Lupus Nephritis
Phase	Phase 3
Patients	460
Primary Outcome Measures	Proportion of subjects achieving protocol-defined CRR
Arms Intervention	Secukinumab 300 mg s.c. Placebo s.c.
Target Patients	Patients with active lupus nephritis (ISN/RPS Class III or IV, with or without co-existing class V features)
Read-out Milestone(s)	2026
Publication	2026

Cosentyx® - IL-17A inhibitor

NCT04930094 GCAPTAIN (CAIN457R12301)

Indication	Giant cell arteritis
Phase	Phase 3
Patients	348
Primary Outcome Measures	Number of participants with sustained remission
Arms Intervention	Experimental: Secukinumab 300 mg Placebo Comparator: Placebo
Target Patients	Patients with Giant Cell Arteritis (GCA)
Read-out Milestone(s)	Primary 2025 Final 2026
Publication	TBD

ianalumab - BAFF-R inhibitor

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 Milestone(s)	2026
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05126277 SIRIUS-LN (CVAY736K12301)

Indication	Lupus Nephritis
Phase	Phase 3
Patients	420
Primary Outcome Measures	Frequency and percentage of participants achieving complete renal response (CRR) [Time Frame: week 72]
Arms Intervention	Arm 1: Experimental - ianalumab s.c. q4w in addition to standard of care (SoC) Arm 2: Experiemntal - ianalumab s.c. q12w in addition to SoC Arm 3: Placebo comparator - Placebo s.c. q4w in addition to SoC
Target Patients	Parients with active Lupus Nephritis
Read-out Milestone(s)	Primary 2027
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05349214 NEPTUNUS-2 (CVAY736A2302)

Indication	Sjögren's syndrome
Phase	Phase 3
Patients	489
Primary Outcome Measures	Change from baseline in EULAR Sjögren Syndrome Disease Activity Index (ESSDAI) score at Week 48 as compared to placebo
Arms Intervention	Arm 1: Experimental - ianalumab exposure level 1 Arm 2: Experimental - ianalumab exposure level 2 Arm 3: Placebo comparator
Target Patients	Patients with active Sjogren's syndrome
Read-out Milestone(s)	Primary 2026
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05350072 NEPTUNUS-1 (CVAY736A2301)

Indication	Sjögren's syndrome
Phase	Phase 3
Patients	268
Primary Outcome Measures	Change from baseline in EULAR Sjögren Syndrome Disease Activity Index (ESSDAI) score at Week 48 as compared to placebo
Arms Intervention	Arm 1: Experimental - ianalumab Arm 2: Placebo comparator
Target Patients	Patients with active Sjogren's syndrome
Read-out Milestone(s)	Primary 2026
Publication	TBD

iscalimab - CD40 inhibitor

NCT03905525 TWINSS (CCFZ533B2201)

Indication	Sjögren's syndrome
Phase	Phase 2
Patients	260
Primary Outcome Measures	Change in EULAR Sjögren's syndrome Disease Activity Index (ESSDAI) score and EULAR Sjögren's syndrome Patient Reported Index (ESSPRI) score
Arms Intervention	Three dose arms of CFZ533 Placebo
Target Patients	Patients with Sjögren's syndrome
Read-out Milestone(s)	2022
Publication	2023

ligelizumab - IgE Inhibitor

NCT04984876 (CQGE031G12301)

Indication	Food allergy
Phase	Phase 3
Patients	486
Primary Outcome Measures	1. Proportion of participants who can tolerate a single dose of ≥ 600 mg (1044 mg cumulative tolerated dose) of peanut protein without dose-limiting symptoms at Week 12
Arms Intervention	Arm 1: ligelizumab 240 mg subcutaneous injection for 52 weeks Arm 2: ligelizumab 120 mg subcutaneous injection for 52 weeks Arm 3: Placebo subcutaneous injection for first 8 weeks and ligelizumab 120 mg subcutaneous injection for 44 weeks Arm 4: Placebo 16 weeks and ligelizumab 120 mg/240 mg subcutaneous injection for 36 weeks Arm 5: Placebo subcutaneous injection for first 8 weeks and ligelizumab 240 mg subcutaneous injection for 44 weeks
Target Patients	Participants with a medically confirmed diagnosis of IgE-mediated peanut allergy
Read-out Milestone(s)	2025
Publication	TBD

LNA043 - ANGPTL3 agonist

NCT04864392 ONWARDS (CLNA043A12202)

Indication	Knee osteoarthritis
Phase	Phase 2
Patients	550
Primary Outcome Measures	Change from baseline in the cartilage thickness of the medial compartment of the knee as assessed by imaging
Arms Intervention	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 Symptomatic knee osteoarthritis
Read-out Milestone(s)	Primary 2024
Publication	TBD

remibrutinib - BTK inhibitor

NCT05030311 REMIX-1 (CLOU064A2301)

Indication	Chronic spontaneous urticaria
Phase	Phase 3
Patients	450
Primary Outcome Measures	Change from baseline in UAS7 (Scenario 1 with UAS7 as primary efficacy endpoint)
Arms Intervention	Arm 1: LOU064 (blinded) LOU064 (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks. Randomized in a 2:1 ratio (arm 1:arm 2). Arm 2: LOU064 placebo (blinded) LOU064 placebo (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally for 28 weeks. Randomized in a 2:1 ratio (arm 1:arm 2).
Target Patients	Adult Chronic Spontaneous Urticaria (CSU) patients inadequately controlled by H1-antihistamines
Read-out Milestone(s)	2024
Publication	TBD

remibrutinib - BTK inhibitor

NCT05032157 REMIX-2 (CLOU064A2302)

Indication	Chronic spontaneous urticaria
Phase	Phase 3
Patients	450
Primary Outcome Measures	1. Change from baseline in UAS7 (Scenario 1 with UAS7 as primary efficacy endpoint) 2. Absolute change in ISS7 an absolute change in HSS7 (Scenario 2 with ISS7 and HSS7 as co-primary efficacy endpoints)
Arms Intervention	Arm 1: LOU064 (blinded) LOU064A (blinded) taken orally b.i.d. for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks Arm 2: LOU064 placebo (blinded) LOU064A placebo (blinded) taken orally for 24 weeks, followed by LOU064 (open-label) taken orally open label for 28 weeks Eligible participants randomized to the treatment arms in a 2:1 ratio (arm 1: arm 2)
Target Patients	Adult participants suffering from chronic spontaneous urticaria (CSU) inadequately controlled by H1-antihistamines in comparison to placebo
Read-out Milestone(s)	2024
Publication	Primary 2025

Neuroscience

branaplam - mRNA splicing modulator

NCT05111249 VIBRANT-HD (CLMI070C12203)

Indication	Huntington`s disease
Phase	Phase 2B
Patients	75
Primary Outcome Measures	1. Reduction (%) of mHTT protein in cerebrospinal fluid (CSF) 2. Number of treatment emergent adverse events and serious adverse events
Arms Intervention	Arm 1: Experimental; Branaplam 56 mg oral solution once weekly Arm 2: Experimental; Branaplam 112 mg oral solution once weekly Arm 3: Experimental; (C) Branaplam 154 mg oral solution once weekly, OR (X) Branaplam 84 mg oral solution once weekly OR (Y) Branaplam 28 mg oral solution once weekly Arm 4: Placebo; Matching placebo oral solution once weekly
Target Patients	Participants with early manifest Huntington's Disease
Read-out Milestone(s)	2025
Publication	TBD

Mayzent® - S1P1,5 receptor modulator

NCT04926818 NEOS (CBAF312D2301)

Indication	Multiple sclerosis, pediatrics
Phase	Phase 3
Patients	180
Primary Outcome Measures	Annualized relapse rate (ARR) in target pediatric participants
Arms Intervention	Arm 1: Experimental ofatumumab - 20 mg injection/ placebo Arm 2: Experimental siponimod - 0.5 mg, 1 mg or 2 mg/ placebo Arm 3: Active Comparator fingolimod - 0.5 mg or 0.25 mg/ placebo
Target Patients	Children/adolescent patients aged 10-17 years old with Multiple Sclerosis (MS). The targeted enrollment is 180 participants with multiple sclerosis which will include at least 5 participants with body weight (BW) ≤40 kg and at least 5 participants with age 10 to 12 years in each of the ofatumumab and siponimod arms. There is a minimum 6 month follow up period for all participants (core and extension). Total duration of the study could be up to 7 years.
Read-out Milestone(s)	2026
Publication	TBD

MIJ821 - NR2B negative allosteric modulator (NAM)

NCT04722666 (CMIJ821A12201)

Indication	Major depressiv disorder with acute suicidal ideation or behavior
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 followed by Placebo 40 minutes IV infusion of 0.9% sodium chloride on Day 15 and Day 29 MIJ821 (mg/kg) very high dose for 40 minutes IV infusion on Day 1 followed by Placebo 40 minutes IV infusion of 0.9% sodium chloride on Day 15 and Day 29
Target Patients	Participants who have suicidal ideation with intent
Read-out Milestone(s)	2023
Publication	TBD

remibrutinib - BTK inhibitor

NCT05147220 REMODEL-1 (CLOU064C12301)

Indication	Multiple sclerosis
Phase	Phase 3
Patients	800
Primary Outcome Measures	Annualized relapse rate (ARR) of confirmed relapses [Core Part]. ARR is the average number of confirmed MS relapses in a year
Arms Intervention	Arm 1: Experimental; Remibrutinib - Core (Remibrutinib tablet and matching placebo of teriflunomide capsule) Arm 2: Active Comparator; Teriflunomide - Core (Teriflunomide capsule and matching placebo remibrutinib tablet) Arm 3: Experimental; Remibrutinib - Extension (Participants on remibrutinib in Core will continue on remibrutinib tablet) Arm 4: Experimental; Remibrutinib - Extension (on teriflunomide in Core) (Participants on teriflunomide in Core will switch to remibrutinib tablet)
Target Patients	Patients with relapsing Multiple Sclerosis
Read-out Milestone(s)	Estimated primary completion 2026
Publication	TBD

remibrutinib - BTK inhibitor

NCT05156281 REMODEL-2 (CLOU064C12302)

Indication	Multiple sclerosis
Phase	Phase 3
Patients	800
Primary Outcome Measures	Annualized relapse rate (ARR) of confirmed relapses
Arms Intervention	Arm 1: Experimental; Remibrutinib - Core Remibrutinib tablet and matching placebo of teriflunomide capsule Arm 2: Active Comparator; Teriflunomide - Core Teriflunomide capsule and matching placebo remibrutinib tablet Arm 3: Experimental: Remibrutinib - Extension Participants on remibrutinib in Core will continue on remibrutinib tablet Arm 4: Experimental: Remibrutinib - Extension (on teriflunomide in Core) Participants on teriflunomide in Core will switch to remibrutinib tablet
Target Patients	Patients with relapsing Multiple Sclerosis
Read-out Milestone(s)	Estimated primary completion 2026
Publication	TBD

Zolgensma® - SMN1 gene replacement therapy

NCT05089656 STEER (COAV101B12301)

Indication	Spinal muscular atrophy (IT administration)
Phase	Phase 3
Patients	125
Primary Outcome Measures	1. Change from baseline in Hammersmith functional motor scale - Expanded (HFMSE) total score at the end of follow-up period 1 in treated patients compared to sham controls in the ≥ 2 to < 18 years age group
Arms Intervention	Arm 1: Experimental OAV101. Administered as a single, one-time intrathecal dose Arm 2: Sham Comparator: Sham control. A skin prick in the lumbar region without any medication.
Target Patients	Patients Type 2 Spinal Muscular Atrophy (SMA) who are ≥ 2 to < 18 years of age, treatment naive, sitting, and never ambulatory
Read-out Milestone(s)	2024
Publication	TBD

Zolgensma® - SMN1 gene replacement therapy

NCT05386680 STRENGTH (COAV101B12302)

Indication	Spinal muscular atrophy (IT administration)
Phase	Phase 3B
Patients	28
Primary Outcome Measures	Number and percentage of participants reporting AEs, related AEs, SAEs, and AESIs [Time Frame: 52 weeks]
Arms Intervention	Experimental: OAV-101 Single intrathecal administration of OAV101 at a dose of 1.2 x 10^14 vector genomes
Target Patients	Participants with SMA who discontinued treatment With Nusinersen or Risdiplam (STRENGTH)
Read-out Milestone(s)	2024
Publication	TBD

Oncology

iptacopan - CFB inhibitor

NCT04558918 APPLY-PNH (CLNP023C12302)

Indication	Paroxysmal nocturnal haemoglobinuria
Phase	Phase 3
Patients	91
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
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
Target Patients	Adult patients with PNH and residual anemia, despite treatment with an intravenous Anti-C5 antibody
Read-out Milestone(s)	Primary 2022
Publication	Risitano AM, et al. Abstract accepted at the European Hematology Association (EHA 2021) congress (study design abstract; accepted for publication only)

iptacopan - CFB inhibitor

NCT04820530 APPOINT-PNH (CLNP023C12301)

Indication	Paroxysmal nocturnal haemoglobinuria
Phase	Phase 3
Patients	40
Primary Outcome Measures	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	Iptacopan (LNP023), taken orally b.i.d. (dosage supplied: 200mg)
Target Patients	PNH patients who are naive to complement inhibitor therapy, including anti-C5 antibody
Read-out Milestone(s)	2022
Publication	Peffault de Latour R, et al. Abstract accepted at the European Hematology Association (EHA 2021) congress (study design abstract; accepted for publication only)

iptacopan - CFB inhibitor

NCT04889430 APPELHUS (CLNP023F12301)

Indication	Atypical haemolytic uraemic syndrome
Phase	Phase 3
Patients	50
Primary Outcome Measures	Percentage of participants with complete TMA response without the use of PE/PI and anti-C5 antibody
Arms Intervention	Single arm open-label with 50 adult patients receiving 200mg oral twice daily doses of iptacopan
Target Patients	Adult patients with aHUS who are treatment naive to complement inhibitor therapy (including anti-C5 antibody)
Read-out Milestone(s)	2024
Publication	TBD

Jakavi® - JAK1/2 inhibitor

NCT03491215 REACH4 (CINC424F12201)

Indication	Acute graft versus host disease
Phase	Phase 2
Patients	45
Primary Outcome Measures	Measurement of PK parameters Overall Response Rate (ORR)
Arms Intervention	Ruxolitinib
Target Patients	Pediatric patients with grade II-IV acute graft vs. host disease after allogeneic hematopoietic stem cell transplantation
Read-out Milestone(s)	2023
Publication	TBD

Jakavi® - JAK1/2 inhibitor

NCT03774082 REACH5 (CINC424G12201)

Indication	Chronic graft versus host disease
Phase	Phase 2
Patients	45
Primary Outcome Measures	Overall Response Rate (ORR)
Arms Intervention	Ruxolitinib 5mg tablets / pediatric formulation
Target Patients	Pediatric subjects with moderate and severe chronic Graft vs. Host disease after allogeneic stem cell transplantation
Read-out Milestone(s)	2023
Publication	TBD

JDQ443 - KRAS inhibitor

NCT05132075 KontRASt-02 (CJDQ443B12301)

Indication	Non-small cell lung cancer, 2/3L
Phase	Phase 3
Patients	360
Primary Outcome Measures	Progression free survival (PFS)
Arms Intervention	Arm 1 Experimental: JDQ443 Arm 2 Active Comparator: Participant will be treated with docetaxel following local guidelines as per standard of care and product labels
Target Patients	Patients with advanced non-small cell lung cancer (NSCLC) harboring a KRAS G12C mutation who have been previously treated with a platinum-based chemotherapy and immune checkpoint inhibitor therapy either in sequence or in combination.
Read-out Milestone(s)	2024
Publication	NA

Kisqali® - CDK4 inhibitor

NCT03701334 NATALEE (CLEE011O12301C)

Indication	Adjuvant treatment of hormone receptor (HR)-positive, HER2-negative, early breast cancer (EBC)
Phase	Phase 3
Patients	5101
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 Milestone(s)	2023
Publication	TBD

NIS793 - TGFβ inhibitor

NCT04935359 daNIS-2 (CNIS793B12301)

Indication	1L metastatic pancreatic ductal Adenocarcinoma
Phase	Phase 3
Patients	501
Primary Outcome Measures	Safety run-in part: Percentage of participants with dose limiting toxicities (DLTs) during the first cycle (4 weeks) of treatment Randomized part: Overall survival (OS)
Arms Intervention	Safety run-in part: NIS793+gemcitabine+nab-paclitaxel Randomized portion of the study: Arm 1: NIS793+gemcitabine+nab-paclitaxel Arm 2: placebo+gemcitabine+nab-paclitaxel
Target Patients	Patients with Metastatic Pancreatic Ductal Adenocarcinoma (mPDAC), first line treatment
Read-out Milestone(s)	Primary: 2024
Publication	NA

Piqray® - PI3K-alpha inhibitor

NCT04208178 EPIK-B2 (CBYL719G12301)

Indication	HER2+ adv breast cancer
Phase	Phase 3
Patients	548
Primary Outcome Measures	Progression-free survival (PFS)
Arms Intervention	Alpelisib + trastuzumab + pertuzumab Trastuzumab + pertuzumab
Target Patients	Patients with HER2-positive advanced breast cancer with a PIK3CA mutation
Read-out Milestone(s)	2025
Publication	TBD

Piqray® - PI3K-alpha inhibitor

NCT04251533 EPIK-B3 (CBYL719H12301)

Indication	Triple negative breast cancer
Phase	Phase 3
Patients	566
Primary Outcome Measures	Progression-free Survival (PFS) for patients with PIK3CA mutant status
Arms Intervention	Alpelisib 300 mg + nab-paclitaxel 100 mg/m² Placebo + nab-paclitaxel 100 mg/m²
Target Patients	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 Milestone(s)	2025
Publication	TBD

Piqray® - PI3K-alpha inhibitor

NCT04729387 EPIK-O (CBYL719K12301)

Indication	Ovarian Cancer
Phase	Phase 3
Patients	358
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 Milestone(s)	2023
Publication	TBD

Pluvicto® - Radioligand therapy target PSMA

NCT04689828 PSMAfore (CAAA617B12302)

Indication	Metastatic castration-resistant prostate cancer, pre-taxane
Phase	Phase 3
Patients	450
Primary Outcome Measures	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
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
Read-out Milestone(s)	Primary Analysis: 2022 Final Analysis: 2025
Publication	TBD

Pluvicto® - Radioligand therapy target PSMA

NCT04720157 PSMAddition (CAAA617C12301)

Indication	Metastatic hormone sensitive prostate cancer
Phase	Phase 3
Patients	1126
Primary Outcome Measures	Radiographic Progression Free Survival (rPFS)
Arms Intervention	Arm 1: 177Lu-PSMA-617 Participant will receive 7.4 GBq (+/- 10%) 177Lu-PSMA-617, once every 6 weeks 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	Patients with metastatic Hormone Sensitive Prostate Cancer (mHSPC)
Read-out Milestone(s)	Primary Analysis: 2024
Publication	TBD

Promacta® - Thrombopoetin receptor agonist

NCT03025698 ESCALATE (CETB115E2201)

Indication	Refractory or relapsed severe aplastic anemia
Phase	Phase 2
Patients	51
Primary Outcome Measures	PK of eltrombopag at steady state in pediatric patients with SAA
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
Target Patients	Pediatric patients from age 1 <18 years with relapsed/refractory SAA or recurrent AA after IST or previously untreated SAA
Read-out Milestone(s)	Primary CSR: 2022 Final CSR: 2025
Publication	Abstract submitted to 64th ASH Annual Meeting and Exposition. 'Eltrombopag in Pediatric Patients with Previously Untreated or Refractory/Relapsed Severe Aplastic Anemia: The Phase II Escalate Trial'

Promacta® - Thrombopoetin receptor agonist

NCT03988608 (CETB115E2202)

Indication	Refractory or relapsed severe aplastic anemia
Phase	Phase 2
Patients	20
Primary Outcome Measures	Hematologic response rate rate up to 26 weeks of treatment
Arms Intervention	Eltrombopag 25 mg film-coated tablets
Target Patients	Chinese patients with refractory or relapsed severe aplastic anemia
Read-out Milestone(s)	Primary CSR: 2021 Interim CSR: 2022 Final CSR:2025
Publication	TBD

Rydapt® - Multi-targeted kinase inhibitor

NCT03591510 (CPKC412A2218)

Indication	Acute myeloid leukemia, pediatrics
Phase	Phase 2
Patients	20
Primary Outcome Measures	Occurrence of dose limiting toxicities Safety and Tolerability
Arms Intervention	Chemotherapy followed by Midostaurin
Target Patients	Newly diagnosed pediatric patients with FLT3 mutated acute myeloid leukemia (AML)
Read-out Milestone(s)	2026
Publication	TBD

sabatolimab - TIM3 antagonist

NCT03946670 STIMULUS MDS-1 (CMBG453B12201)

Indication	Myelodysplastic syndrome
Phase	Phase 2
Patients	120
Primary Outcome Measures	Complete Remission (CR) rate and Progression Free Survival (PFS)
Arms Intervention	Experimental: Sabatolimab (MBG453) + hypomethylating agents Placebo comparator: Placebo + hypomethylating agents
Target Patients	Adult subjects with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as per IPSS-R criteria
Read-out Milestone(s)	2022 ClinicalTrial.gov dates for reference: Primary Completion: 29-Apr-2022; Secondary Completion: 10-Aug-2024
Publication	

sabatolimab - TIM3 antagonist

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 Milestone(s)	2023
Publication	TBD

sabatolimab - TIM3 antagonist

NCT04266301 STIMULUS-MDS2 (CMBG453B12301)

Indication	Myelodysplastic syndrome
Phase	Phase 3
Patients	500
Primary Outcome Measures	Overall survival
Arms Intervention	Sabatolimab 800 mg + azacitidine 75 mg/m2 Sabatolimab 800 mg + azacitidine 75 mg/m2 + placebo
Target Patients	Patients with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as Per IPSS-R, or Chronic Myelomonocytic Leukemia-2 (CMML-2)
Read-out Milestone(s)	2024
Publication	TBD

Scemblix® - BCR-ABL inhibitor

NCT04971226 ASC4FIRST (CABL001J12301)

Indication	Chronic myeloid leukemia, 1st line
Phase	Phase 3
Patients	402
Primary Outcome Measures	Major Molecular Response (MMR) at week 48
Arms Intervention	Arm 1: asciminib 80 mg QD Arm 2: Investigator selected TKI including one of the below treatments: - Imatinib 400 mg QD - Nilotinib 300 mg BID - Dasatinib 100 mg QD - Bosutinib 400 mg QD
Target Patients	Patients with newly diagnosed philadelphia chromosome positive chronic myelogenous leukemia in chronic phase
Read-out Milestone(s)	2024
Publication	TBD

Tabrecta® - Met inhibitor

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 Milestone(s)	Primary 2022 Final: 2024
Publication	TBD

TNO155 - SHP2 inhibitor

NCT03114319 (CTNO155X2101)

Indication	Solid tumors (single agent)
Phase	Phase 1
Patients	255
Primary Outcome Measures	Number of participants with adverse events Number of participants with dose limiting toxicities
Arms Intervention	Drug: TNO155 Drug: TNO155 in combination with EGF816 (nazartinib)
Target Patients	Adult patients with advanced solid tumors in selected indications
Read-out Milestone(s)	2023
Publication	TBD

Other

Ophthamology

Beovu® - VEGF Inhibitor

NCT04278417 (CRTH258D2301)

Indication	Diabetic retinopathy
Phase	Phase 3
Patients	706
Primary Outcome Measures	Change from Baseline in BCVA
Arms Intervention	Arm1: RTH258 (brolucizumab) 6 mg/50uL Arm2: Panretinal photocoagulation laser initial treatment followed with additional PRP treatment as needed
Target Patients	Patients with proliferative diabetic retinopathy
Read-out Milestone(s)	2024
Publication	TBD

libvatrep - TRPV1 antagonist

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 Milestone(s)	2023
Publication	2023

UNR844 - Reduction of disulfide bonds

NCT04806503 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 Milestone(s)	2022: Primary endpoint- when all patients have completed the 3 months treatment period (Actual) 2023: Final analysis -Study completion (all patients have completed 9 months pots treatment period)
Publication	H1-2023

Global Health

Adakveo® - P-selectin inhibitor

NCT03474965 SOLACE-Kids (CSEG101B2201)

Indication	Sickle cell disease, pediatrics
Phase	Phase 2
Patients	100
Primary Outcome Measures	PK/PD and safety of SEG101 at 5 mg/kg
Arms Intervention	SEG101 (crizanlizumab) at a dose of 5 mg/kg by IV infusion ± Hydroxyurea/Hydroxycarbamide
Target Patients	Pediatric SCD patients with VOC
Read-out Milestone(s)	H2-2021 (pediatric patients ≥12 year old) 2024 (pediatric patients <12 year old)
Publication	1. Matthew M. Heeney, David C. Rees, Mariane de Montalembert, Isaac Odame, R. Clark Brown, Yasser Wali, Thu Thuy Nguyen, Du Lam, Raquel Merino Herranz, Julie Kanter; Study Design and Initial Baseline Characteristics in Solace-Kids: Crizanlizumab in Pediatric Patients with Sickle Cell Disease. Blood 2020; 136 (Supplement 1): 22–24. doi: https://doi.org/10.1182/blood-2020-137081 2. Matthew M. Heeney, David C. Rees, Mariane De Montalembert, Isaac Odame, R. Clark Clark Brown, Yasser Wali, Thu Thuy Nguyen, Du Lam, Nadege Pfender, Julie Kanter; Initial Safety and Efficacy Results from the Phase II, Multicenter, Open-Label Solace-Kids Trial of Crizanlizumab in Adolescents with Sickle Cell Disease (SCD). Blood 2021; 138 (Supplement 1): 12. doi: https://doi.org/10.1182/blood-2021-144730

cipargamin - PfATP4 inhibitor

NCT04675931 KARISMA (CKAE609B12201)

Indication	Malaria severe
Phase	Phase 2
Patients	252
Primary Outcome Measures	Percentage of participants achieving at least 90% reduction in Plasmodium falciparum (P. falciparum) at 12 hours [Time Frame: Day 1 (12 Hours)]
Arms Intervention	Arm 1: experimental, IV KAE609 Dose regimen 1 Arm 2: experimental, IV KAE609 Dose regimen 2 Arm 3: experimental, IV KAE609 Dose regimen 3 Arm 4: active comparator, IV Artesunate Arm 5: Coartem, Standard of care
Target Patients	Patients with Malaria, severe
Read-out Milestone(s)	2024
Publication	TBD

Coartem® - PGH-1

NCT04300309 CALINA (CCOA566B2307)

Indication	Malaria, uncomplicated (<5kg patients)
Phase	Phase 3
Patients	44
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 Milestone(s)	Primary outcome measure: 2023
Publication	TBD

ganaplacide - Imidazolopiperazines derivative

NCT04546633 KALUMI (CKAF156A2203)

Indication	Malaria, uncomplicated
Phase	Phase 2
Patients	292
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 6 months to < 18 years old
Read-out Milestone(s)	2023
Publication	TBD

Biosimilars

aflibercept - VEGF inhibitor

NCT04864834 Mylight (CSOK583A12301)

Indication	Ophthalmology indication (as originator)
Phase	Phase 3
Patients	460
Primary Outcome Measures	Best-corrected visual acuity (BCVA) will be assessed using the ETDRS testing charts at an initial distance of 4 meters. The change from baseline in BCVA in letters is defined as difference between BCVA score between week 8 and baseline
Arms Intervention	Arm 1 Biological: SOK583A1 (40 mg/mL) Arm 2 Biological: Eylea EU (40 mg/mL)
Target Patients	Patients with neovascular age-related macular degeneration
Read-out Milestone(s)	2023
Publication	tbd

Abbreviations

aBC	Advanced breast cancer
AD	Atopic Dermatitis
Adj.	Adjuvant
AIH	Autoimmune hepatitis
aHUS	atypical Hemolytic Uremic Syndrome
ALL	Acute lymphoblastic leukemia
ALS	Amyotrophic lateral sclerosis
AMI	Acute myocardial infarction
AML	Acute myeloid leukemia
aNHL	Agressive non-Hodgkin's lymphoma
AS H2H	Ankylosing spondylitis head-to-head study versus adalimumab
BC	Breast cancer
C3G	C3 glomerulopathy
CCF	Congestive cardiac failure
CINDU	Chronic inducible urticaria
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
ESCC	Esophageal squamous-cell carcinoma
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
jPsA/ERA	Juvenile psoriatic arthritis / enthesitis-related arthritis
LVEF	Left ventricular ejection fraction
mCRPC	Metastatic castration-resistant prostate cancer
MDR	Multi-drug resistant
MDS	Myelodysplastic syndrome
MS	Multiple sclerosis
NASH	Non-alcoholic steatohepatitis
nHCM	Non-obstructive hypertrophic cardiomyopathy
nr-axSpA	Non-radiographic axial spondyloarthritis
NSCLC	Non-small cell lung cancer
PEF	Preserved ejection fraction
PedPsO	Pediatric psoriasis
PNH	Paroxysmal nocturnal haemoglobinuria
PsA	Psoriatic arthritis
PROS	PIK3CA related overgrowth spectrum
RA	Rheumatoid arthritis
rMS	Relapsing multiple sclerosis
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

References

Entresto®	
1	IQVIA National Prescription Audit as of Sep ’22.
2	Eligible patients defined as prevalent HFrEF patients within each market’s label. G7: US, CA, JP, DE, FR, IT, UK/
3	Zhang et al., ESC Heart Failure 2020; 7: 3841
4	Proudfoot et al., Int J Cardiol. 2021; 331:164
5	Including, but not limited to, the recent 2022 AHA/ACC/HFSA Guideline for the Management of Heart Failure (Heidenreich et al., J Am Coll Cardiol. 2022)
6	Approved indications differ by geography. Examples include “indicated to reduce the risk of cardiovascular death and hospitalization for HF in adult patients with CHF. Benefits are most clearly evident in patients with LVEF below normal.” (US) HFrEF (EU) HFrEF and HTN (China and JP)
7	Novartis estimate of patients on treatment across all indications