Q4 2022 Results
Investor presentation

NOVARTIS | Reimagining Medicine

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Vas Narasimhan, M.D.

Chief Executive Officer

Company overview

STRATEGY

Novartis delivers robust core operating income growth and core margin expansion; achieves important innovation milestones

Growth, cc 1

Group sales Q4 +3% (**FY +4%**)
IM sales Q4 +3% (**FY +4%**); US IM sales Q4 +7%
Sandoz sales Q4 +0% (**FY +4%**)

Innovation 3

Pluvicto EU approval for mCRPC post-taxane
Iptacopan Ph3 APPOINT-PNH met primary endpoint
Pluvicto Ph3 PSMAfore in mCRPC pre-taxane met primary endpoint
Ianalumab Ph3 initiated in wAIHA; Ph3 initiating in 1L ITP and 2L ITP

Productivity, cc 2

Group core operating income Q4 +15% (**FY +8%**)
IM core operating income Q4 +14% (**FY +8%**)
IM core margin Q4 36.4%, +3.5%pts (**FY 36.9%**)
Sandoz core operating income Q4 -18% (**FY -1%**)
SG&A savings of ~USD 1.5bn to be fully embedded by 2024[2]

ESG 4

Sustainability Linked Bond: Significant progress towards 2025 targets
Innovative Therapies in LMICs: ~1.2m patients; +26.3% vs. 2021
Novartis flagship programs: ~31m patients vs. 2025 target ~23m
Ratings: CDP - AA score[1]; ATMI: Leaders group 10th consecutive year

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. mCRPC – metastatic castration-resistant prostate cancer. wAIHA – warm autoimmune hemolytic anemia. ITP – immune thrombocytopenia. LMIC – low and middle income countries. CDP – carbon disclosure project. ATMI – Access to Medicine Index. 1. One of only two pharma companies with AA rating. 2. Relating to streamlined organizational model.

STRATEGY

New Novartis[1]: Our focused strategy

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

Our focus

5 core Therapeutic Areas[2]

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**

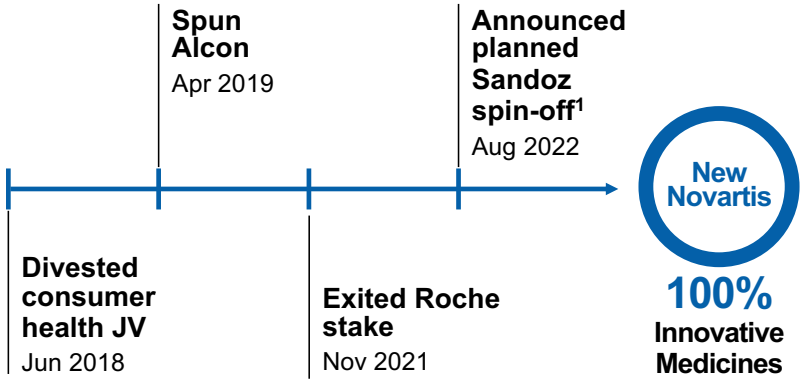
Build trust with **society**

1. New Novartis here and in the rest of the presentation refers to Novartis assuming planned Sandoz spin-off. Subject to final Novartis AG BoD and shareholder approval (EGM). 2. Other TAs opportunistically.

STRATEGY

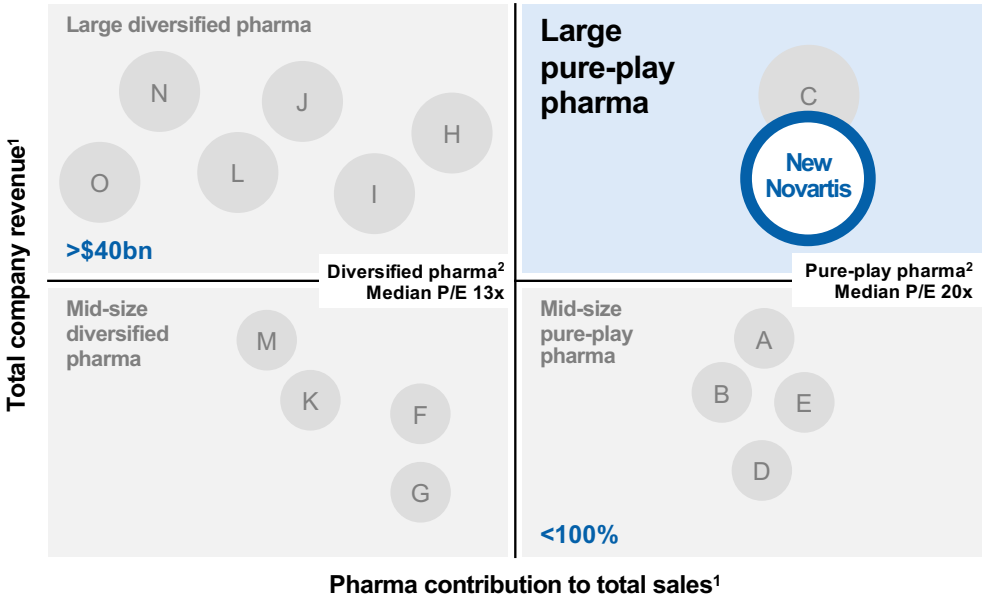
As a pure-play Innovative Medicines company, New Novartis is uniquely positioned to leverage its scale, strengths and expertise

Novartis is on track to become a pure-play Innovative Medicines company



Simplified organizational model allowing for greater focus, leveraging scale and expertise

Illustrative

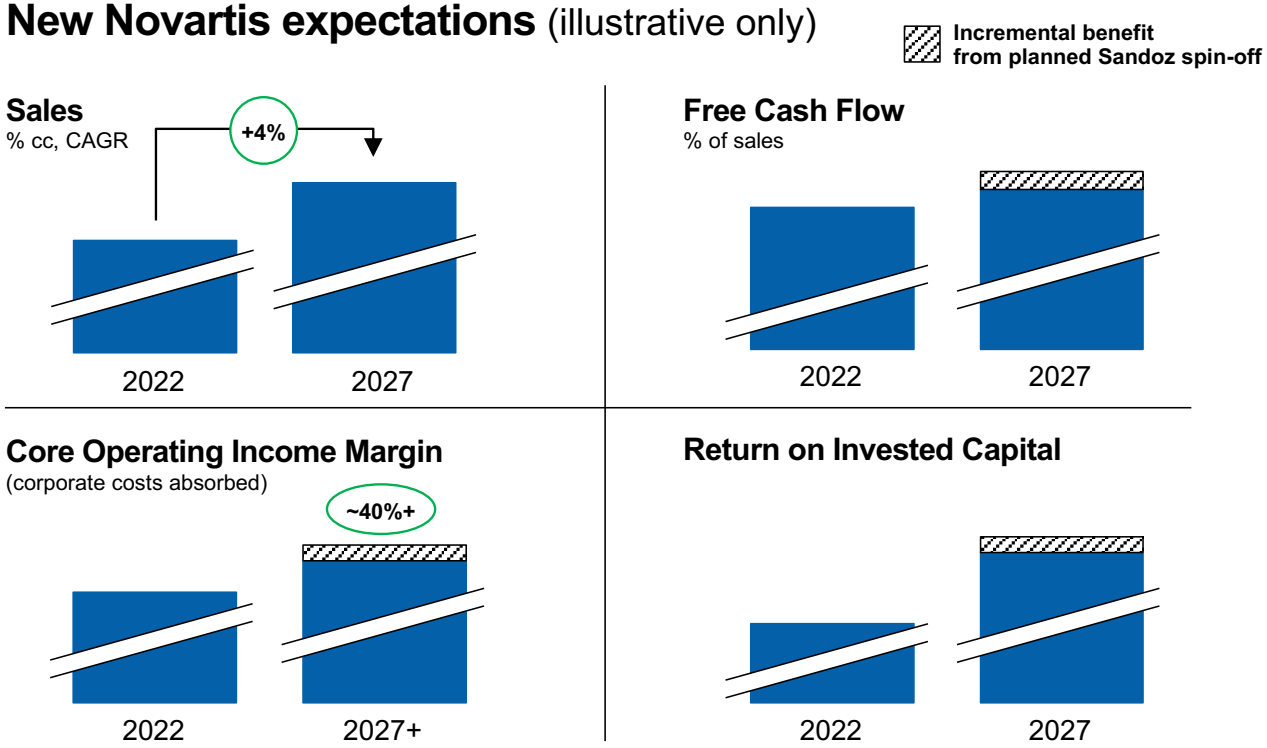


1. Company filings and FactSet. 2. Median P/E (Bloomberg, current year).

STRATEGY

New Novartis expected to continue delivering improved financials

New Novartis expectations (illustrative only)



Expected to grow sales, core margin and FCF (% of sales)

Margin targets include absorbing corporate costs

Planned Sandoz **spin-off will result in incremental growth** for:

- Core operating income margin
- FCF (% of sales)
- Return on invested capital

Remain committed to capital allocation priorities, with growing (CHF) annual dividend

STRATEGY

Focused on five core Therapeutic Areas that have the largest growth potential and leverage our existing assets/expertise

Select examples[1]

	Cardiovascular	Immunology	Neuroscience	Solid Tumors	Hematology
Disease areas (selected)	▪ Heart failure & hypertension ▪ Atherosclerosis	▪ Psoriasis ▪ Psoriatic arthritis ▪ Spondylitis/Spondylarthritis ▪ Hidradenitis suppurativa ▪ CSU ▪ Sjögren's / SLE / LN	▪ Multiple sclerosis ▪ Spinal muscular atrophy ▪ Neurodegeneration, including Parkinson's, ALS	▪ Breast and Women's cancer ▪ Prostate cancer ▪ Lung cancer	▪ Non-Hodgkin's Lymphoma ▪ Non-malignant hematological - Immune thrombocytopenia ▪ Acute myeloid leukemia / Myelodysplastic syndrome
Commercial assets	Entresto (sacubitril/valsartan), LEQVIO	Cosentyx (secukinumab), ILARIS (canakinumab), Xolair (omalizumab)	Kesimpta (ofatumumab), Zolgensma, MAYZENT (siponimod) tablets, Aimovig[2], GILENYA (fingolimod)	KISQALI (ribociclib), PLUVICTO, Tafinlar (dabrafenib) + Mekinist (trametinib), PIQRAY (alpelisib) tablets	KYMRIAH (tisagenlecleucel), SCEMBLIX (asciminib), PROMACTA (eltrombopag), JAKAVI (ruxolitinib)
Pipeline assets and opportunities	**Iptacopan (LNP023)** C3G, IgAN	**Cosentyx** Multiple indications	**Remibrutinib (LOU064)** MS	**Kisqali** Adjuvant HR+/HER2- BC	**Iptacopan (LNP023)** PNH, aHUS
	Pelacarsen[3] (TQJ230) CVRR-Lp(a)	**Remibrutinib (LOU064)** CSU	**OAV101** SMA IT	**JDQ433** NSCLC	**Ianalumab (VAY736)** Multiple indications
	Leqvio CVRR-LDLC	**Ianalumab (VAY736)** Sjögren's, SLE, LN	**DLX313[4]** Parkinson's	**NIS793** 1L mPDAC / 1L mCRC	**YTB323** Non-Hodgkin's Lymphoma
	XXB750 HFpEF, rHT	**Ligelizumab (QGE031)** Food Allergy		**Pluvicto** Prostate cancer	
FY Sales USD[5]	**4.8bn**	**7.3bn**	**5.1bn**	**4.7bn**	**6.5bn**

1. TA-x (incl. Ophtha, Resp and other assets) not included in the above list. 2. Aimovig® is commercialized by Novartis ex-US/Japan. 3. Pelacarsen is licensed from Ionis Pharmaceuticals, Inc. 4. DLX313 is Novartis program name for UCB0599. 5. FY 2022 sales for entire Therapeutic Area.

STRATEGY

Our capital allocation priorities are shareholder-focused, allowing for flexibility in strategic investments and capital distribution

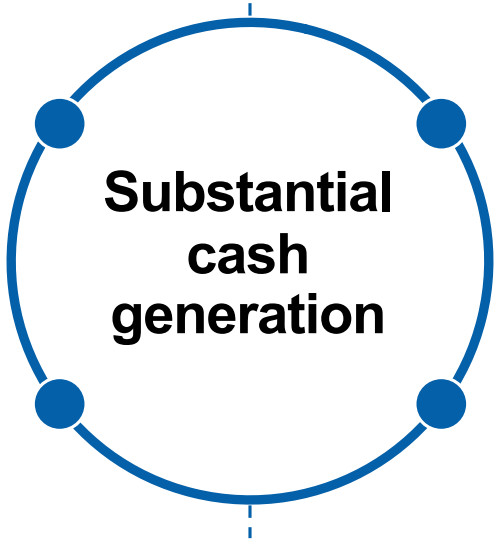
Investing in the business

Investments in organic business

USD 9.1bn R&D 2022[1]
USD 1.2bn capital investments 2022

Value-creating bolt-ons

USD 30bn (approx.) 2018-2022



Returning to shareholders

USD 59bn distributed 2018-2022

Growing annual dividend in CHF

USD 7.5bn paid out in 2022
Proposed DPS: **+3.2% CHF; +3.9% USD**
No rebasing post planned Sandoz spin-off

Share buybacks

USD 15bn ongoing
USD 4.9bn to be executed[2]

Planned Sandoz spin-off is expected to have limited impact on our credit rating, providing continued flexibility for future capital allocations

1. Core R&D and cap-ex actuals 2022. 2. As of December 31, 2022.

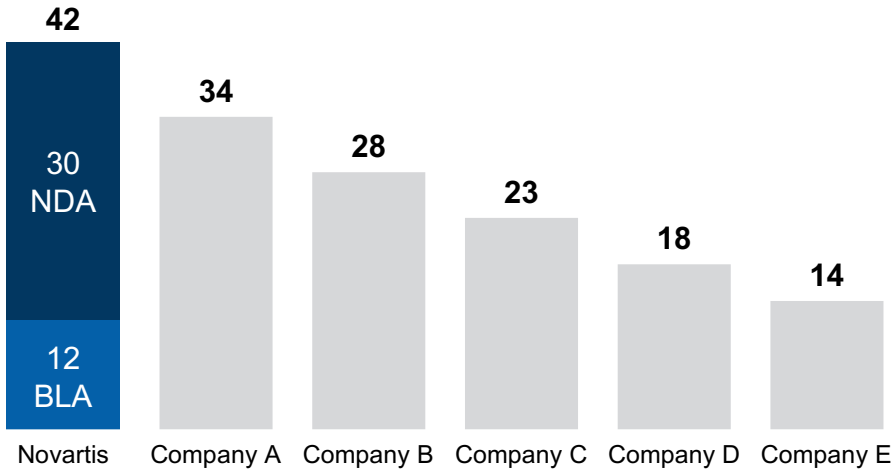
INNOVATION

Prioritizing pipeline to high-value innovative medicines

Refining our proven development engine with greater focus on asset value and improving R&D productivity

Proven development engine

Total NME approvals by company (1999-2021)[1]



☑ Industry leader across first-in-class approved NMEs[2]

Improving R&D productivity

1. Clear TA strategy with disease area prioritization
2. Early assets with integrated development plans, until submission
3. Ongoing tracking and evaluation of asset progression/value
4. End-to-end governance with clear processes and ownership

Expected outcomes

- ☑ Improved overall success rate (discovery to approval)
- Cycle time reduction
- Increased asset value

1. US FDA NME approvals. 2. FDA: BCG analysis (2017-2021).

INNOVATION

Key near-term readouts (2023) for high-value medicines...

Key assets* with submission enabling readouts in 2023

Kisqali® ●●●

NATALEE trial in adjuvant breast cancer testing broad patient population (anatomical stage II and III[1]), with final analysis expected in **H2 2023**

Iptacopan ●●●

APPOINT-PNH trial in treatment-naive patients positive readout; detailed data presentation in **2023**

PNH FDA submission planned **H1 2023**

APPLAUSE-IgAN Ph3 readout[2] planned in **H2 2023**

APPEAR-C3G Ph3 readout planned in **H2 2023**

Pluvicto® ●●

PSMAfore trial in mCRPC (post-ARDT, pre-taxane) positive readout; detailed data presentation planned in **2023**

FDA regulatory submission planned **H2 2023**

* Unprobabilized peak sales of all asset indications in late-stage development: ● > USD 1bn ●● > USD 2bn ●●● > USD 3bn

1. Based on AJCC prognostic staging. 2. 9 months analysis potentially supporting US Subpart H filing.

INNOVATION

… set to increase in 2024-2025 timeframe

Key assets* with submission enabling readouts in 2024-2025

Pelacarsen ●●●
CVRR
Readout and submission in **2025**

Iptacopan ●●●
Additional readouts/submissions in **2025/2026+**

Ianalumab ●●●
1L and 2L ITP readouts in **2025** with submission in **2026**
Additional hematology and immunology indications **2026+**

Pluvicto® ●●
mHSPC
Readout and submission in **2024**

Remibrutinib ●●
CSU
Readout and submission in **2024**

OAV-101 ●●
SMA IT
Readout in **2024**; submission in **2025**

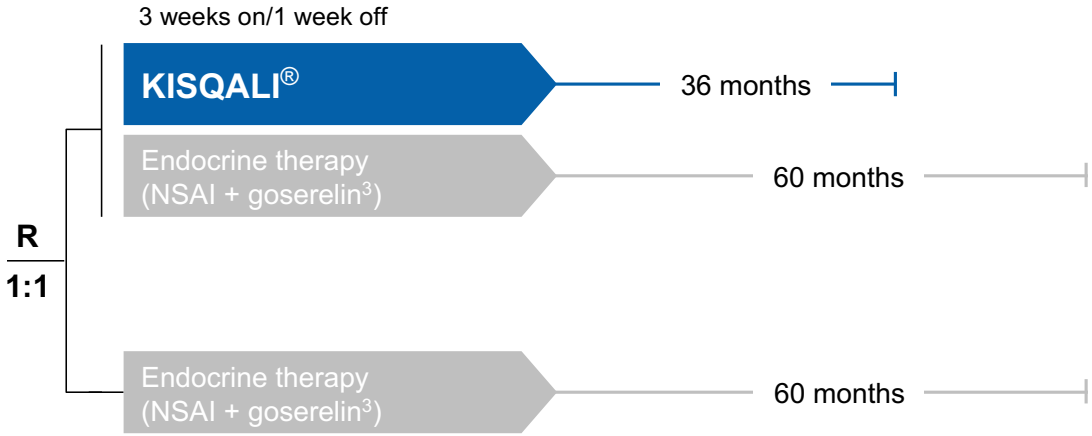
Scemblix® ●●
1L CML-CP
Readout in **2024**; submission in **2025**

* Unprobabilized peak sales of all asset indications in late-stage development: ● > USD 1bn ●● > USD 2bn ●●● > USD 3bn

INNOVATION

Kisqali® – NATALEE study continues following first interim analysis; final readout expected H2 2023

NATALEE: HR+/HER2- pre- and post-menopausal, early breast cancer Stage II & III | N=5000



Patient population: 218K patients with early breast cancer in US + EU[1]

NATALEE differentiation

Broad population including **anatomical Stage II and III**[2]

Longer treatment duration of **3 vs. 2 years** (monarchE)

Lower dose (400mg/day) to potentially improve tolerability and adherence without compromising efficacy

Study status

Final analysis planned at 500 iDFS events, expected H2 2023

1st interim analysis at 70% of events (completed, study continues)

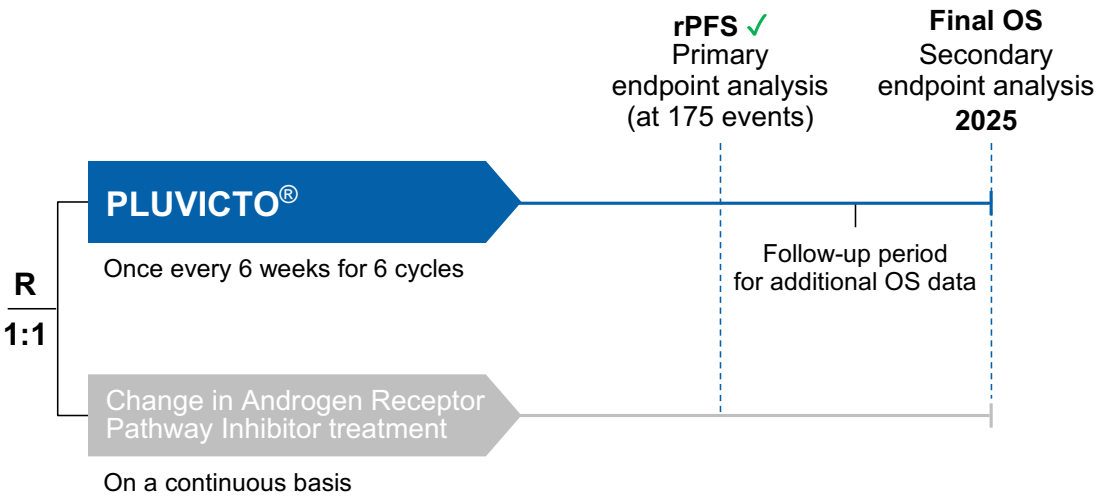
2nd interim analysis at 85% of events

NSAI – non-steroidal aromatase inhibitor. 1. eBC Patient - Adjuvant Breast Cancer Opportunity Assessment June 2020. 2. Based on AJCC prognostic staging. 60% Stage III and 40% Stage II patients in study. Stage II vs. Stage III is a stratification factor. The trial did not require Ki-67% or other CDx for patient identification or stratification, but Ki-67% is part of the statistical analysis plan. 3. In pre-menopausal women and in men.

INNOVATION

Pluvicto® – PSMAfore study demonstrated statistically significant and clinically meaningful radiographic PFS benefit; filing H2 2023

PSMAfore: Patients with mCRPC who progressed while on ARPI treatment | N=450



Study status

Met the primary endpoint (rPFS) – statistically significant and clinically meaningful

First PSMA-targeted RLT to demonstrate clinical benefit in patients with mCRPC who have not yet received taxane-based chemotherapy

FDA regulatory **submission planned for H2 2023**, to include OS data as aligned with FDA

Addressing a significant unmet need in earlier lines of metastatic prostate cancer

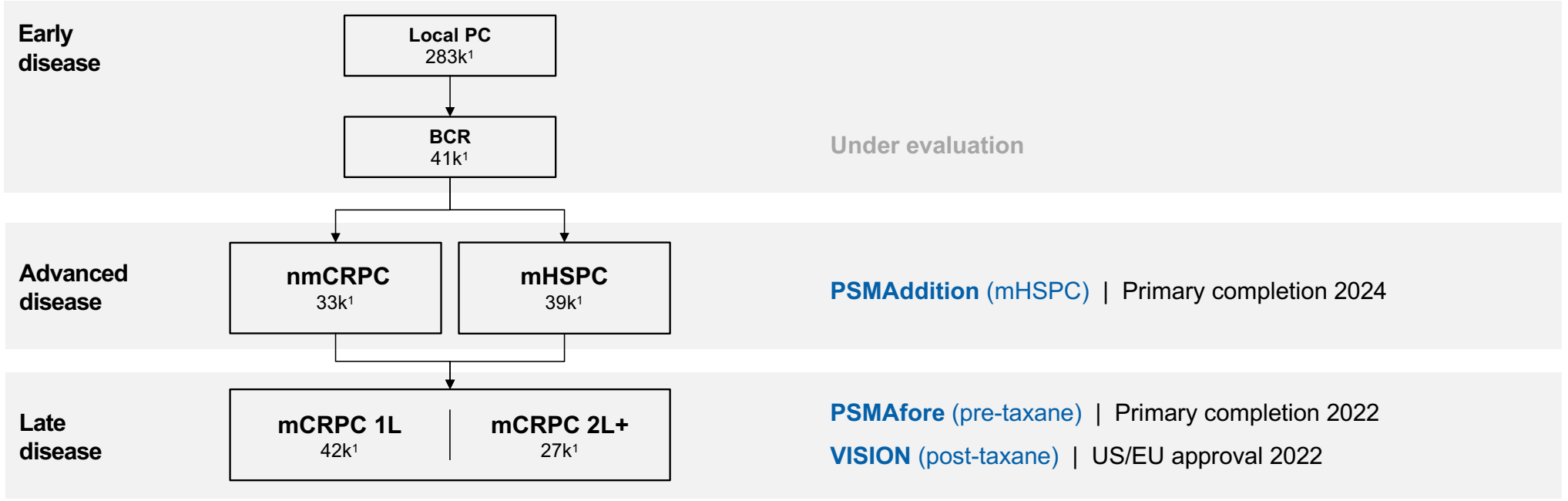
rPFS – radiographic progression free survival. OS – overall survival. mCRPC – metastatic castration-resistant prostate cancer.

INNOVATION

Expanding Pluvicto® development program to address significant unmet need in earlier lines and stages of prostate cancer

Our ongoing clinical development plan for Pluvicto® in prostate cancer

Prostate cancer incidence in US, in patients per year

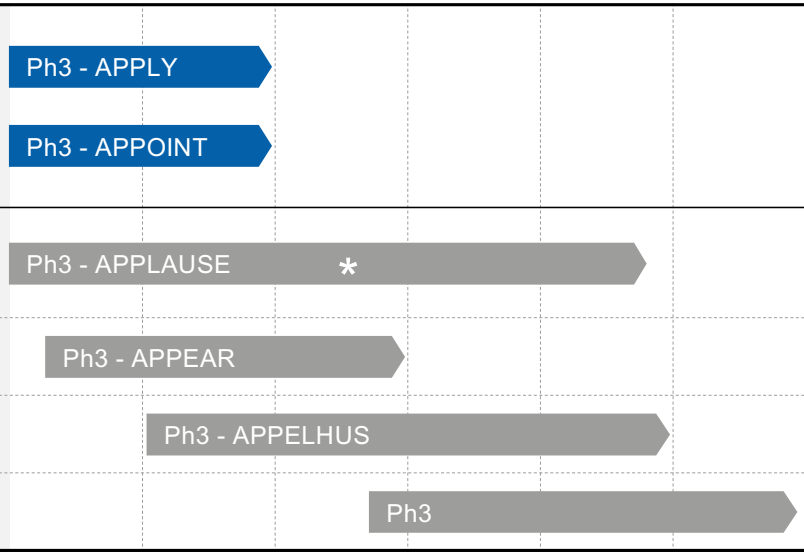


PC – prostate cancer. BCR – biochemical recurrence. nmCRPC – non-metastatic castration-resistant prostate cancer. mHSPC – metastatic hormone-sensitive prostate cancer. mCRPC – metastatic castration-resistant prostate cancer.
1. Source: Kantar 2023 US Prostate Cancer Incidence, as of 30-Jan-2023

INNOVATION

Iptacopan – provides the opportunity to redefine care across multiple complement-driven conditions[1]

First-in-class, oral, selective factor B inhibitor, targeting complement system proximally via alternative pathway[1]



Key updates	US prevalence Thousands
☑ Superior to SoC for both primary endpoints in patients with residual anemia despite SoC ☑ Achieved clinically meaningful increases in Hb levels in treatment-naive patients with PNH	<10
▶ 9 months analysis (H2 2023) potentially provides basis for US Subpart H filing on proteinuria reduction	185[2]
▶ Submission enabling readout H2 2023	<10
▶ Submission enabling readout in 2025	<10
▶ Ph3 start in H2 2023	<10

Additional ongoing early-stage (Ph2) activities in Lupus Nephritis, iAMD and Immune thrombocytopenia

PNH – paroxysmal nocturnalhemoglobinuria. IgAN – immunoglobulin A nephropathy. C3G – complement 3 glomerulopathy. aHUS – atypical hemolytic uremic syndrome. iAMD – intermediate age-related macular degeneration. IC-MPGN – immune complex membranoproliferative Glomerulonephritis. *9 months readout may support US submission for accelerated approval. 1. Phase 3 studies initiated or planned; additional indications are being explored. 2. Estimated ~46-55k number of patients at high risk of progression with proteinuria > 1g/day (~25-30%).

INNOVATION

Iptacopan demonstrated superiority over anti-C5 in adult PNH patients with residual anemia despite anti-C5 treatment...

APPLY-PNH: Randomized, active-comparator controlled Ph3 trial in adult PNH patients with residual anemia, despite treatment with an intravenous anti-C5 antibody

	Endpoints	Observed	Population estimate[2]	
		Iptacopan vs. SoC	Iptacopan vs. SoC	Difference
PRIMARY	**Increase from baseline in Hb of ≥ 2 g/dL** in the absence of RBC transfusions	**51**/60[1] vs. **0**/35	82.3% vs. 2.0%	**80.3%** (95% CI 71.3, 87.6) P<0.0001[3]
PRIMARY	**Hb ≥ 12 g/dL** in the absence of RBC transfusions	**42**/60[1] vs. **0**/35	68.8% vs. 1.8%	**67.0%** (95% CI 56.3, 76.9) P<0.0001[3]
SECONDARY	**Transfusion avoidance**	**60**/62 vs. **14**/35	96.4% vs. 26.1%	**70.3%** (95% CI 52.6, 84.9) P<0.0001[3]
SECONDARY	**Clinical breakthrough hemolysis**	2/62 vs. 6/35	Rate ratio (95% CI) of 0.10 (0.02, 0.61) means **10-fold lower** rate of annualized clinical breakthrough hemolysis	

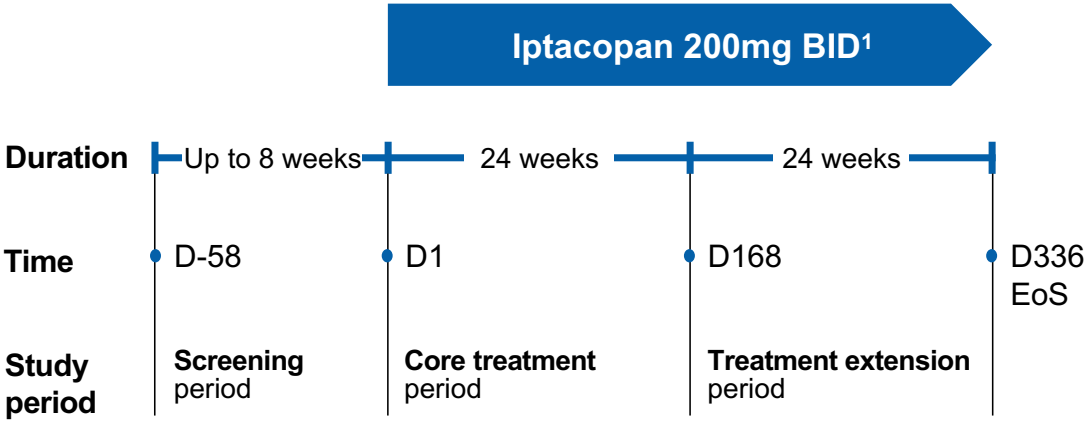
Improvements across a range of other secondary endpoints: Increasing Hb, reducing patient-reported fatigue and reticulocyte count, maintaining low LDH

PNH – paroxysmal nocturnalhemoglobinuria. 1. 2/62 patients in the iptacopan arm had missing data between Days 126 and 168 so were not evaluable based on observed data. 2. Marginal proportions reflect the population average probability of a patient meeting the endpoint criteria. 3. P values are two-sided and unadjusted.

INNOVATION

… and showed clinically meaningful increases in Hb levels for treatment-naive adult patients with PNH

APPOINT-PNH: Single-arm Ph3 trial in adult patients with PNH with hemolysis (LDH > 1.5x ULN) and anemia (Hb < 10g/dL) naive to complement inhibitors



Study status

Met primary endpoint of proportion of patients achieving a sustained increase in Hb of ≥ 2g/dL, in the absence of transfusions, at 24 weeks

Safety profile consistent with previously reported data

Data to be presented at upcoming medical meeting

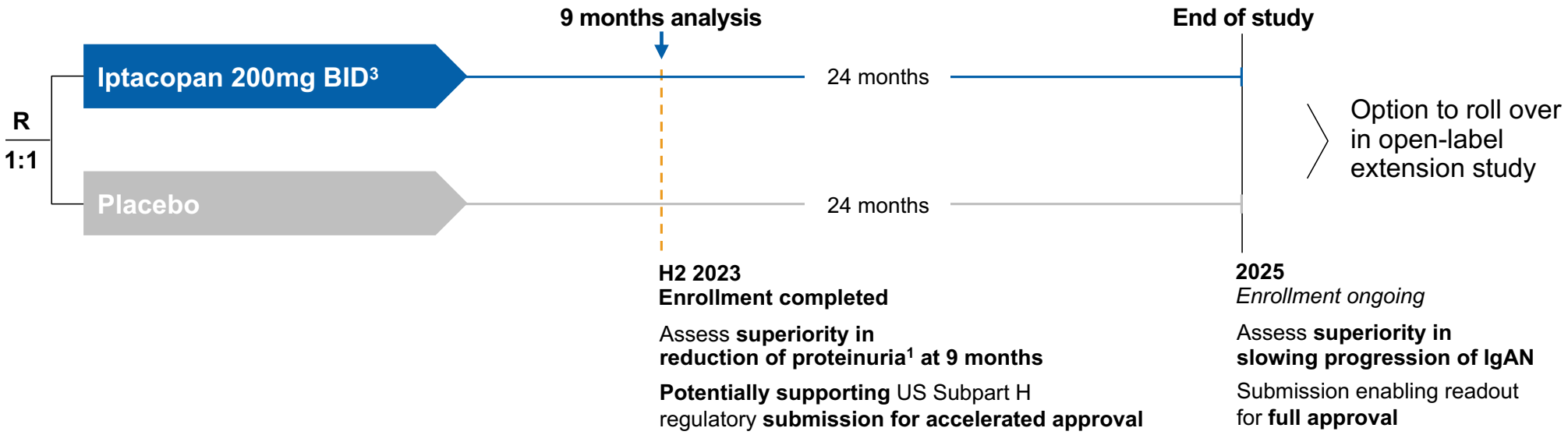
Iptacopan has the potential to be practice-changing in PNH

PNH – paroxysmal nocturnalhemoglobinuria. 1. BID – twice daily.

INNOVATION

Upcoming 9 months analysis from IgAN Ph3 study (APPLAUSE) could potentially support US Subpart H filing

APPLAUSE-IgAN: Biopsy-confirmed patients with IgAN at risk of progression with elevated proteinuria (UPCR[1] ≥1g/g) despite being on stable background therapy[2]



1. UPCR (urine protein-to-creatinine ratio) from 24-h urine collection. 2. Including at least maximally tolerated dose of ACEi/ARB for at least 90 days. 3. BID – twice daily.

INNOVATION

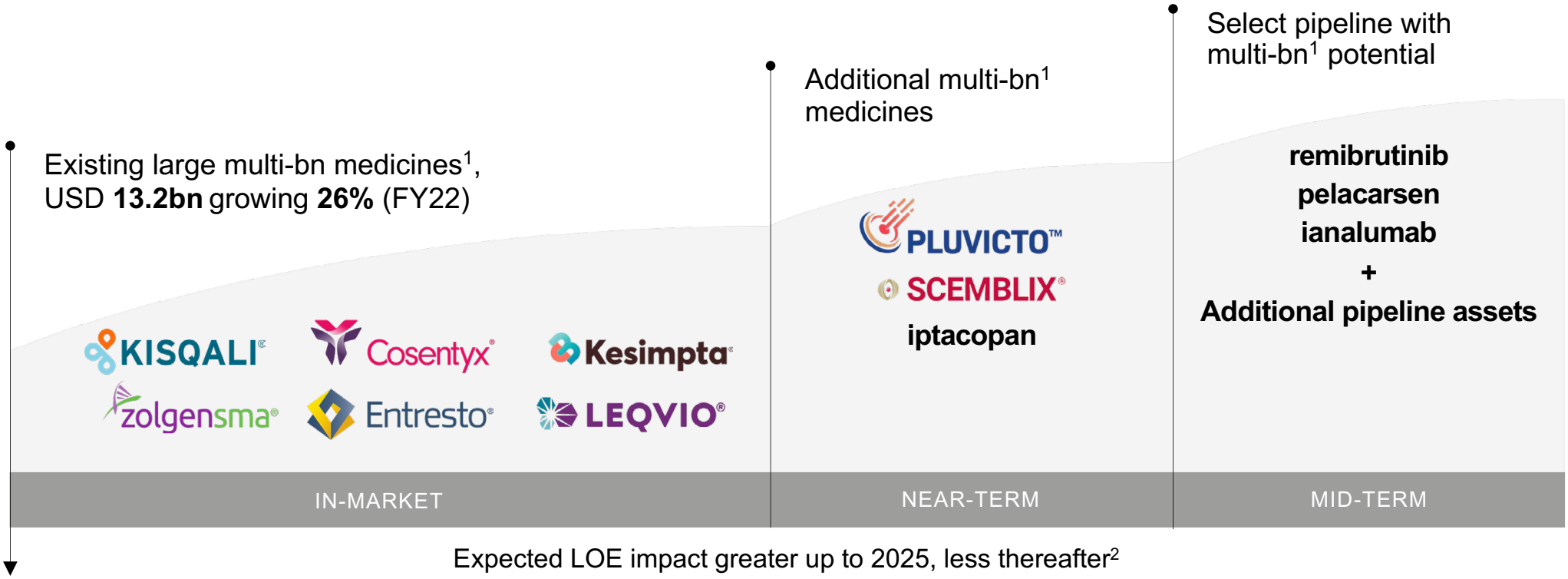
Selected early stage assets with the potential to deliver high-impact medicines for patients

Compound	Therapeutic area	MoA	Indication(s)	Current phase	Next milestone
XXB750	Cardiovascular	NPR1 agonist	**Hypertension** Heart failure	Ph2b	Ph2b readout 2024
YTB323	Hematology	CD19 RAPID CART	**1L HR LBCL**	Ph2	Data readout(s) 2024/2025
	Immunology		Multiple indications being explored	-	-
AAA603	Solid Tumors	Radioligand therapy targeting GRPR	**Breast cancer** Glioblastoma	Ph1	Ph1 readout – H2 2023
PPY988	Ophthalmology	Gene therapy - Complement factor I modulation	**Geographic atrophy**	Ph2b	Topline data – 2024
DLX313	Neuroscience	a-Synuclein inhibitor	**Parkinson's**	Ph2a	Topline data – 2024

NPR1 – natriuretic peptide receptor 1. CART – chimeric androgen receptor T. LBCL – large B-cell lymphoma. GRPR – gastrin releasing peptide receptor.

GROWTH

Confidence in near and mid-term growth underpinned by multi-bn existing medicines and pipeline



1. Potential USD sales. 2. For forecasting purposes, we assume Entresto LOE in 2025.

GROWTH

2022 driven by particularly strong performance from Entresto®, Kesimpta®, Kisqali® and Pluvicto®

FY sales[1]

	Sales USD million	Growth vs. PY USD million	Growth vs. PY cc
Entresto *	4,644	1,096	37%
Kesimpta *	1,092	720	200%
KISQALI *	1,231	294	38%
PLUVICTO *	271	271	nm
SCEMBLIX *	149	142	nm
LEQVIO *	112	100	nm
Tafinlar + Mekinist	1,770	77	11%
MAYZENT	357	76	32%
ILARIS	1,133	74	15%
PROMACTA	2,088	72	9%
Cosentyx *	4,788	70	5%

2022:
Strong growth (+60% cc)

2023 and beyond:
Expect continued strong growth

Expect LCM to accelerate growth

* Please see subsequent slides

Constant currencies (cc) is a non-IFRS measure; explanation of non-IFRS measures can be found on page 50 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.
nm – not meaningful. LCM – life cycle management.

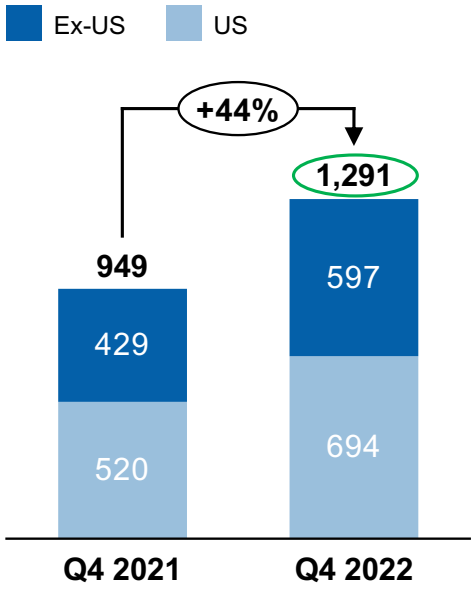
GROWTH

Entresto® delivers strong growth across all geographies

Entresto®

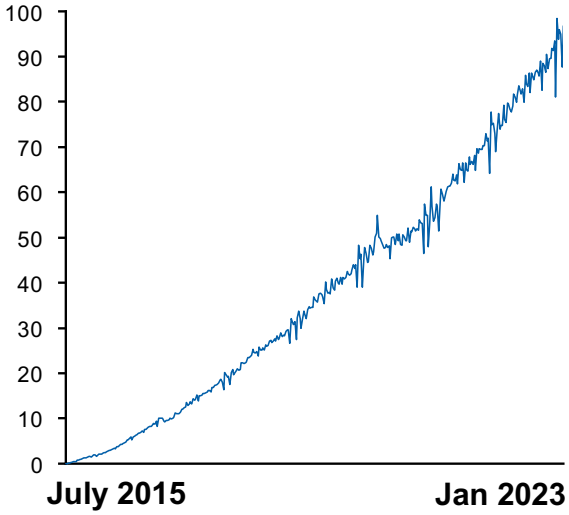
Sales evolution

USD m, % cc



US weekly TRx[2]

Total prescriptions (000)



Strong Q4 momentum across geographies

US: NBRx +16% vs PY, ~1.2m TRx in Q4[1]

EU: continued growth in HFrEF

China/Japan: significant contribution from HTN[2]

FY sales **4.6bn (+37% cc, +1.1bn)**

Confidence in future growth[3]

US/EU: further penetration in CHF

Strong clinical profile and RW data in heart failure

China/Japan: launch momentum in HTN

TRx – total prescriptions. HFrEF – heart failure with reduced ejection fraction. CHF – chronic heart failure. RW – real world. HTN – hypertension. 1. IQVIA National Prescription Audit. 2. 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). HTN is not an approved indication in the US. 3. For forecasting purposes, we assume Entresto® LOE in 2025.

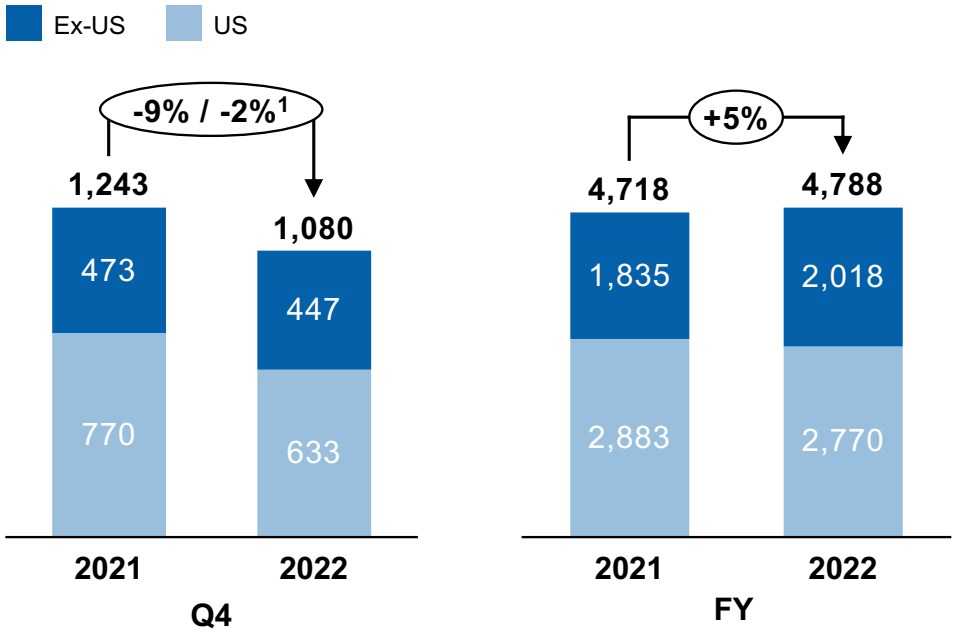
GROWTH

Cosentyx® Q4 sales impacted mainly by a revenue deduction true-up related to prior quarters in the US and China COVID lockdowns

Cosentyx®

Sales evolution

USD m, % cc



Q4 performance

US: -18%/-6%[1]. Impacted by a RD true-up relating to prior quarters in 2022 driven primarily by a higher Medicaid channel mix

Ex-US: +5% (cc), growth driven by core indications

China: Impacted by COVID and stock compensation provision for 2023 NRDL re-listing

Volume continues to grow across key geographies

FY sales **USD 4.8bn (+5% cc) driven by ex-US (+20% cc)**

Future growth mainly driven by LCM

2023: Expect sales broadly in line with PY (H2 growth and H1 decline[2])

2024+: LCM anticipated to drive next phase of growth

RD – revenue deduction. NRDL – national reimbursement drug list. LCM – life cycle management. 1. US Cosentyx® sales growth was impacted by a revenue deduction true-up, which was related to prior quarters in 2022. Global sales declined -9% in Q4 vs. PY (decline would have been -2% without the true-up). US sales declined -18% in Q4 vs. PY (decline would have been -6% without the true-up). 2. Expect continued RD dynamics in US.

GROWTH

Cosentyx® – life cycle management to drive growth from 2024

Peak sales potential including all existing and new indications > USD 7bn

Cosentyx®

Indication — Potential

Hidradenitis Suppurativa (HS)
Giant Cell Arteritis (GCA)
Lupus Nephritis (LN)
2mL AI/PFS & IV expansion

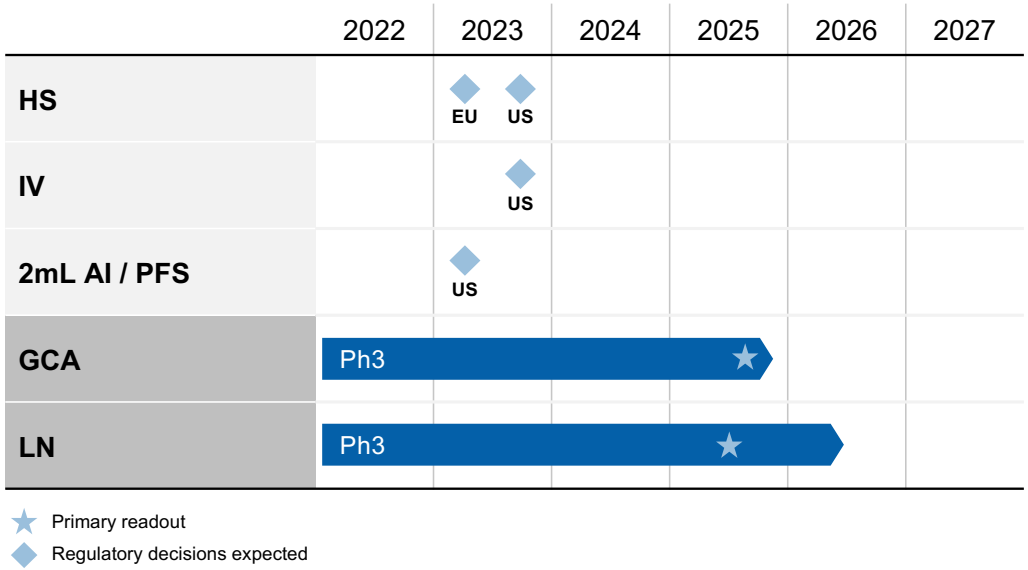
Additional Ph3 indication programs under assessment

●○○ <USD 1bn ●●○ USD 1-2bn ●●● >USD 2bn

Diagnosed population[1]

Indication	Patients	Unmet need
HS	>400K	Debilitating skin disease with significant QoL impact
GCA	>480K	Eye-sight threatening vasculitis in elderly
LN	>130K	Major cause of morbidity and mortality in SLE patients

Life cycle development milestones



HS – hidradenitis suppurativa. GCA – giant cell arthritis. LN – lupus nephritis. AI – auto injector. PFS – prefilled syringe. IV – intravenous formulation (rheumatology indications). 1. Total diagnosed population G6 countries.

NOVARTIS | Reimagining Medicine

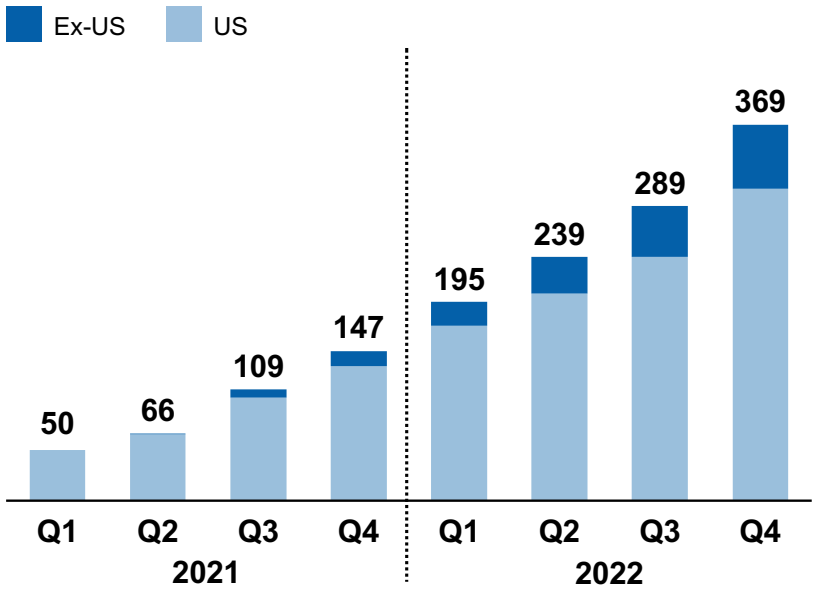
GROWTH

Kesimpta® shows strong growth driven by a compelling product profile

Kesimpta

Sales evolution

USD m, % cc



Global sales +28% (cc) vs. Q3, driven by US[1]

TRx +116% vs. PY (market +1%)[1,2]

NBRx +43% vs. PY (market -8%)[1,2]

B-cell NBRx share about 50% of MS market[1,2]

Kesimpta® B-cell NBRx share ~30%[1,2]

Adding ~80-90 new writers/month[1,3]

Fast initiation: <6 days for 80% patients[1,4]

FY sales **USD 1.1bn (+200% cc)**

Confident in future growth

Powerful efficacy: 9/10 patients with NEDA-3 at year 4[5]

Convenience: 1 minute a month dosing from home or anywhere[6]

TRx – total prescriptions. NBRx – new to brand prescription. NEDA – No Evidence of Disease Activity 1. Refers to US unless otherwise stated. 2. December 2022, IQVIA NPA (Kesimpta®) and IQVIA NPA adjusted by NSP (all others). B-cell therapies as portion of MS market in NBRx. 3. Data on file. 4. Measured as days between prescription and dispense of first dose. Refers to patients in Kesimpta® Patient Support Program. Data on file. 5. Kuhle, et al. Poster presented at ECTRIMS, 26–28 October, 2022. 6. The initial dosing period consists of 20 mg subcutaneous doses at Weeks 0, 1 and 2, thereafter once a month. Patient must take pen out of the refrigerator 15-30 minutes before self-administering.

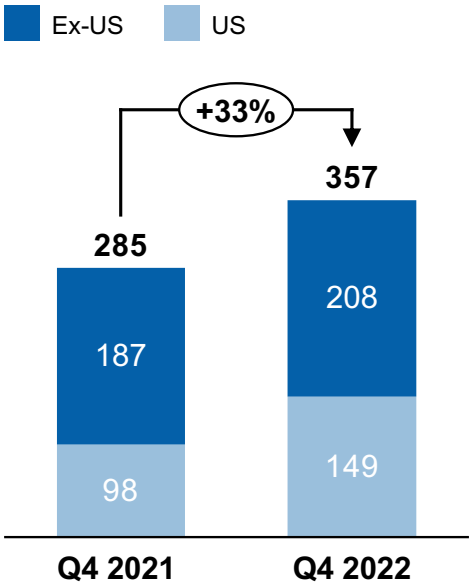
GROWTH

Kisqali® delivers strong growth across all geographies based on increasing recognition of overall survival and quality of life benefits

KISQALI®

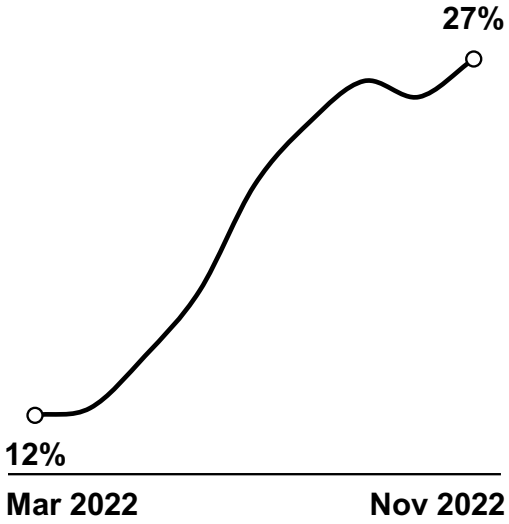
Sales evolution

USD m, % cc



US mBC NBRx share[1]

R3M, %



FY sales **USD 1.2bn (+38% cc)**

NBRx share 27%[1]

NCCN update[2]: The only Category 1 treatment for 1L mBC with AI; Category 1 for 1L with fulvestrant

RIGHT Choice Ph2 study: **Kisqali® doubled median PFS** in patients with aggressive HR+/HER2− mBC compared to CT[3]

NATALEE final analysis expected **H2 2023**

HARMONIA H2H (vs. Ibrance®) recruiting

Approved in China for pre/peri-menopausal HR+/HER2- mBC

mBC – metastatic breast cancer. R3M – rolling 3 months. H2H – head-to-head. 1. Of CDK4/6 mBC market, US Nov'22 R3M. 2. NCCN Guidelines updated as of 27-Jan-2023. 3. RIGHT Choice evaluated Kisqali® plus endocrine therapy (ET) vs. combination chemotherapy (CT) in 1L pre- and perimenopausal patients with aggressive forms of HR+/HER2− mBC, including patients with visceral crisis. Data presented at SABCS: mPFS 24.0 vs. 12.3 months; HR=0.54; p=.0007.

NOVARTIS | Reimagining Medicine

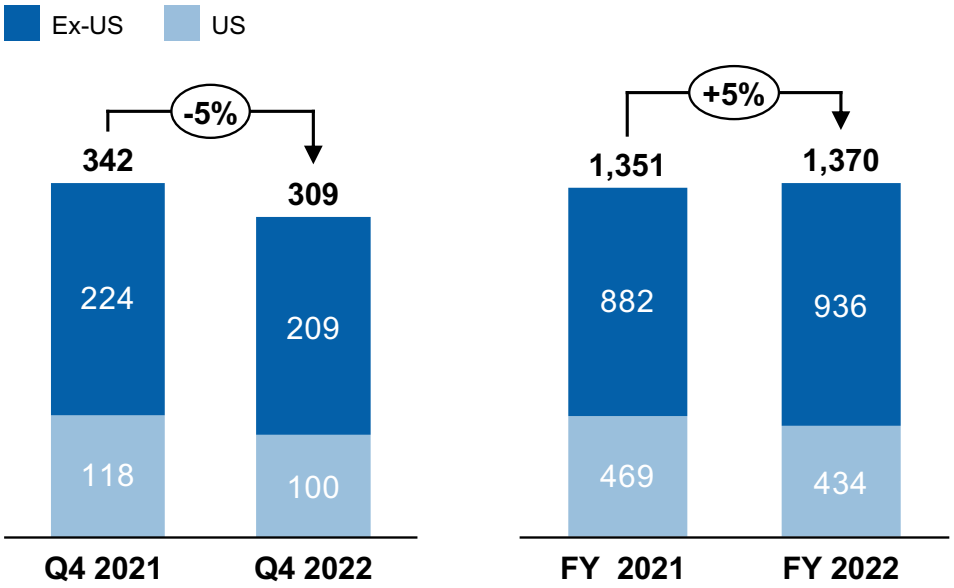
GROWTH

Zolgensma® maintains leading share in patients with SMA <2 years of age[1]; Q4 growth impacted by prior year prevalent boluses

zolgensma®

Sales evolution

USD m, % cc



Markets **mainly incident** patient population

Maintaining leader share **(>90% US)** in <2 years

Q4 sales **decline due to prior year prevalent boluses in ex-US markets (Europe/Canada)**

FY sales **USD 1.4bn (+5% cc)**

Continued efforts to **increase newborn screening ex-US** (45% in Europe; >99% in US)

IT development on track – Ph3 STEER and STRENGTH enrolling

1. Based on US data.

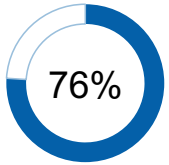
GROWTH

Leqvio® – we are continuing to progress the launch in Q4...

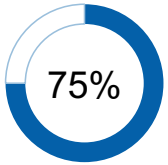
LEQVIO®

Addressing non-clinical barriers

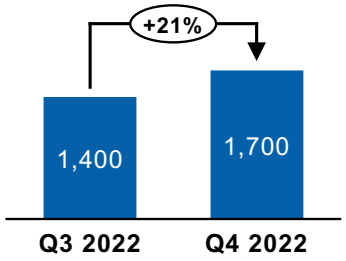
Access
Patients covered **at or near label**[1]



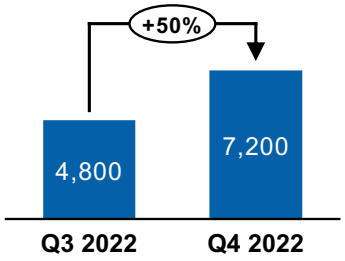
Adherence
Patients coming for **2nd dose within <95 days**[4]



Adoption
Facilities[2] having **ordered Leqvio®**



Adoption
HCPs[3] with **Leqvio® experience**



FY sales **USD 112 million**

ORION-3 data and publication

- Strong long-term safety profile
- Effective/sustained reductions in LDL-C over 4 years
- At any time throughout the trial ~80% of patients reached LDL-C <70mg/dL

Ph3 **secondary prevention** CVRR studies (ORION-4 / V2P) **ongoing**

Ph3 **primary prevention** CVRR study expected start **H1 2023**

HCP – healthcare professional. V2P – VICTORION-2-PREVENT. 1. As of January 3, 2022. 2. Either an alternate site of care or a physician practice. 3. Either prescribe Leqvio® to a patient based on service center data, data on file or have ordered through Free Trial Offer program. 4. Refers to average duration in between doses. Based on IQVIA and data shared by infusion management and ambulatory infusion center companies. *Leqvio® is administered initially, again at 3 months, and then once every 6 months. **Novartis has obtained global rights to develop, manufacture and commercialize Leqvio® under a license agreement with Alnylam Pharmaceuticals.

GROWTH

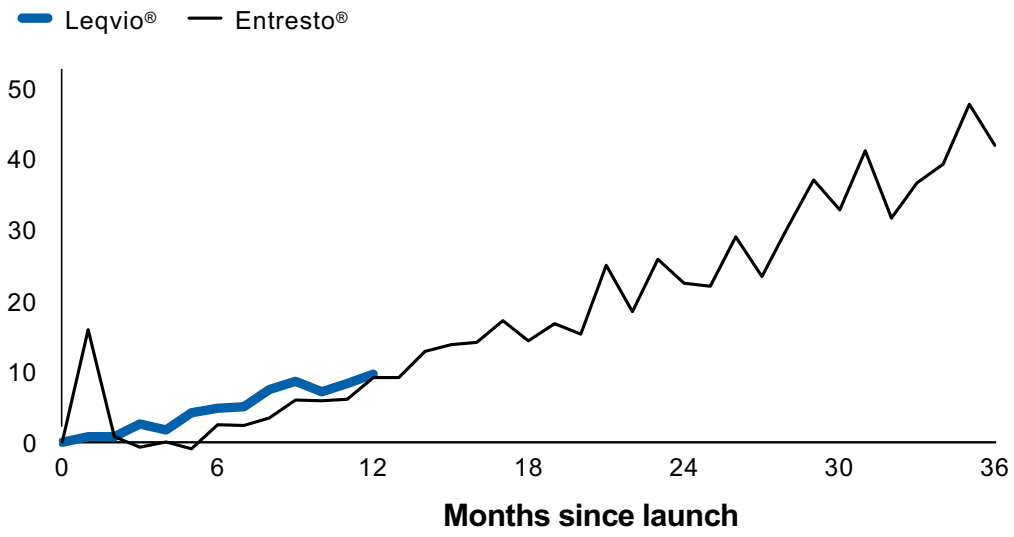
… expect steady ramp in 2023 similar to Entresto® US trajectory

LEQVIO®

Leqvio® in line with initial Entresto® launch

US monthly sales evolution

USD m



2023 focus

US: Accelerate adoption

Enable new facilities to order Leqvio® (breadth)

Accelerate growth among prescribers (depth)

Support HCPs with acquisition/reimbursement process

Expect gradual conversion from Free Trial offer

Ex-US: Continue rollout

Expand prescriber breadth in **UK**

Anticipate regulatory approval in additional geographies including **China** (Q4 2023)

Prepare for anticipated launches in **China/Japan/Spain**

*Leqvio® is administered initially, again at 3 months, and then once every 6 months. **Novartis has obtained global rights to develop, manufacture and commercialize Leqvio® under a license agreement with Alnylam Pharmaceuticals.

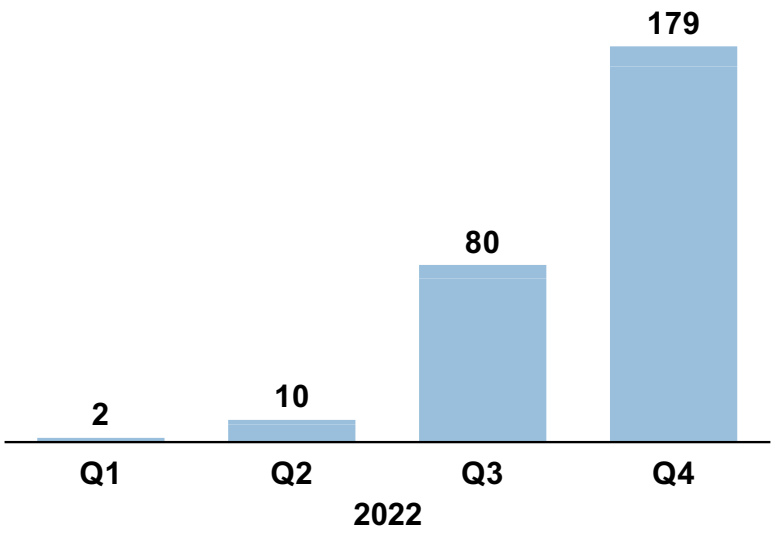
GROWTH

Pluvicto® rapid uptake in US reflects very strong demand, driven by significant unmet need and impressive clinical profile

PLUVICTO™

Sales evolution

Global sales, USD m



FY sales **USD 271 million** (almost entirely US)

NBRx share 18% in post-taxane mCRPC

160+ unique accounts in US treated with Pluvicto in 2022

More than 75% of insured lives covered
(across Medicare, Medicaid and private payers)

Permanent A code effective in October

Approved in EU for mCRPC post-taxane

NBRx – new to brand prescriptions. mCRPC – metastatic castration-resistant prostate cancer.

GROWTH

Pluvicto® manufacturing capacity is set to significantly expand in 2023 with bringing Millburn and Indianapolis on-line

PLUVICTO™

Expected coverage by end 2023

Manufacturing site[1]:	Ivrea	Millburn	Indianapolis	Zaragoza
Commercial	US, EU, RoW[2]	Canada, US[3]	US[3]	-
Clinical	EU, RoW	Canada, US, EU	-	EU, RoW[2]

Targeting capacity of at least 250k doses annually for 2024+

1. Additional manufacturing sites in Asia under evaluation. 2. Dependent on regulatory approval. 3. Expected in H2 2023.

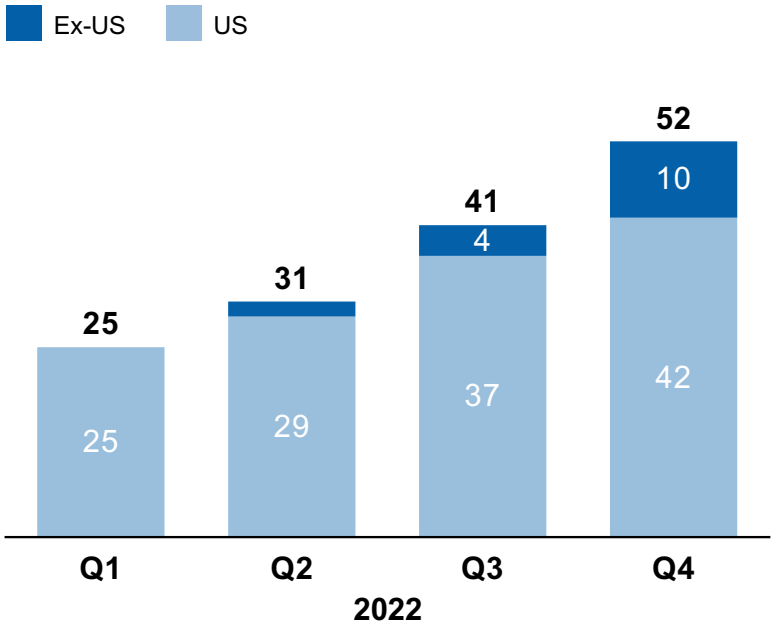
GROWTH

Scemblix® launch off to a strong start

SCEMBLIX® (asciminib) 20 mg, 40 mg tablets

Sales evolution

USD m



FY sales **USD 149 million**

US sales driven by patients resistant/intolerant to other TKIs; CML 3L+ **NBRx share at 29%**[1]

Global rollout ongoing with approval in 40 countries; access pathways in 9, negotiations ongoing in 30+

ASCEND IIT presented at ASH, showing promising preliminary results and consistent tolerability in 1L CML

ASC4FIRST (1L registrational study) completed enrollment ahead of plan, readout expected 2024

1. IQVIA: US Oct 2022 rolling three months 3L+ new patient start share.

Harry Kirsch
Chief Financial Officer

Financial review and 2023 guidance

FINANCIAL PROFILE

2022 financial results in line with guidance; ahead on Core OpInc

Group full year guidance (Q3 earnings October 2022)
In cc

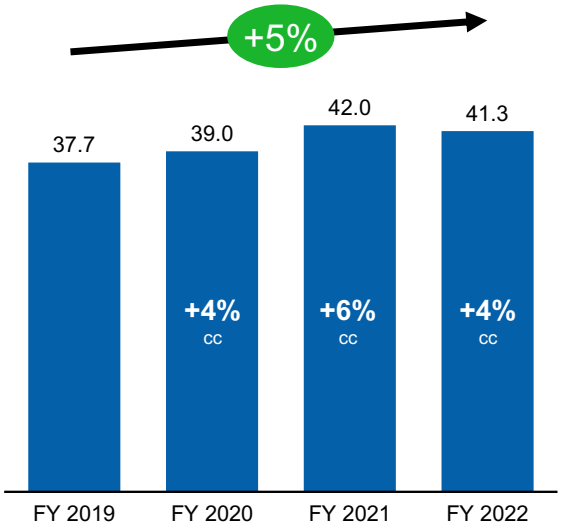
		FY 2022 vs. PY
Innovative Medicines	Sales to **grow mid single digit**	+4%
	Core OpInc to **grow mid to high single digit, ahead of sales**	+8%
Sandoz	Sales to **grow low to mid single digit**	+4%
	Core OpInc to **grow low single digit**	-1%
Group	Sales to **grow mid single digit**	+4%
	Core OpInc to **grow mid single digit**	**+8%**

FINANCIAL PROFILE

2022 performance continues our track record of consistent top-line growth and core margin expansion for Innovative Medicines (IM)

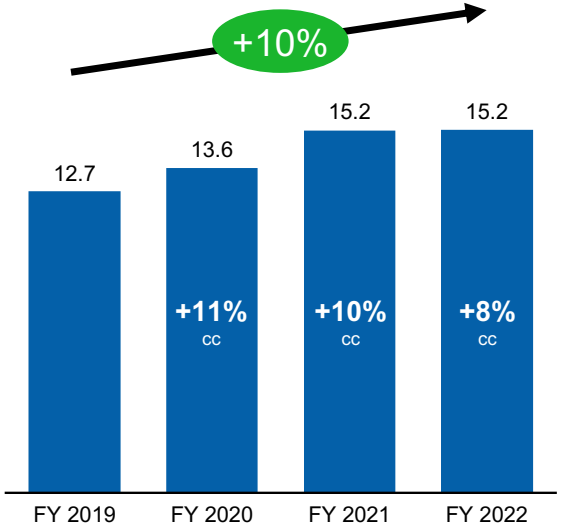
IM Sales[1]

USD bn, % CAGR cc



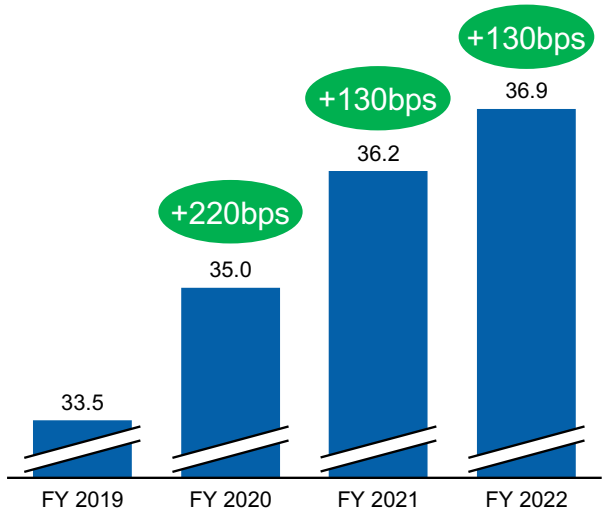
IM Core OpInc[1]

USD bn, % CAGR cc



IM Core Margin[1]

(%), growth bps cc



1. 2022 FY Sales growth +4% cc and -2% USD, 2022 FY Core OpInc growth +8% cc and 0% USD.

FINANCIAL PROFILE

Solid Q4 and FY performance

Group[1] USD million	Q4 2022	Change vs. PY % USD	Change vs. PY % cc	FY 2022	Change vs. PY % USD	Change vs. PY % cc
Net Sales	12,690	-4	3	50,545	-2	4
Core Operating income	4,030	6	15	16,665	0	8
Operating income	1,949	-24	-14	9,197	-21	-13
Net Income	1,466	-91	-90	6,955	-71	-67
Growth ex. prior year Roche income		*-12*	*2*		*-20*	*-9*
Core EPS (USD)	1.52	9	19	6.12	-3	6
Growth ex. prior year Roche income		*12*	*23*		*5*	*14*
EPS (USD)	0.69	-91	-89	3.19	-70	-66
Growth ex. prior year Roche income		*-8*	*7*		*-17*	*-7*
Free Cash Flow	3,552	17		11,945	-10	
Growth ex. prior year Roche dividend		*17*			*-6*	

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 50 of the Condensed Financial Report. A table showing the Q4 2022 and FY 2022 key figures excluding Roche can be found on page 3 and 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 59 of the Condensed Interim Financial Report.

FINANCIAL PROFILE

Novartis proposes 26th consecutive dividend increase to the AGM: 3.20 CHF / share[1]

2022

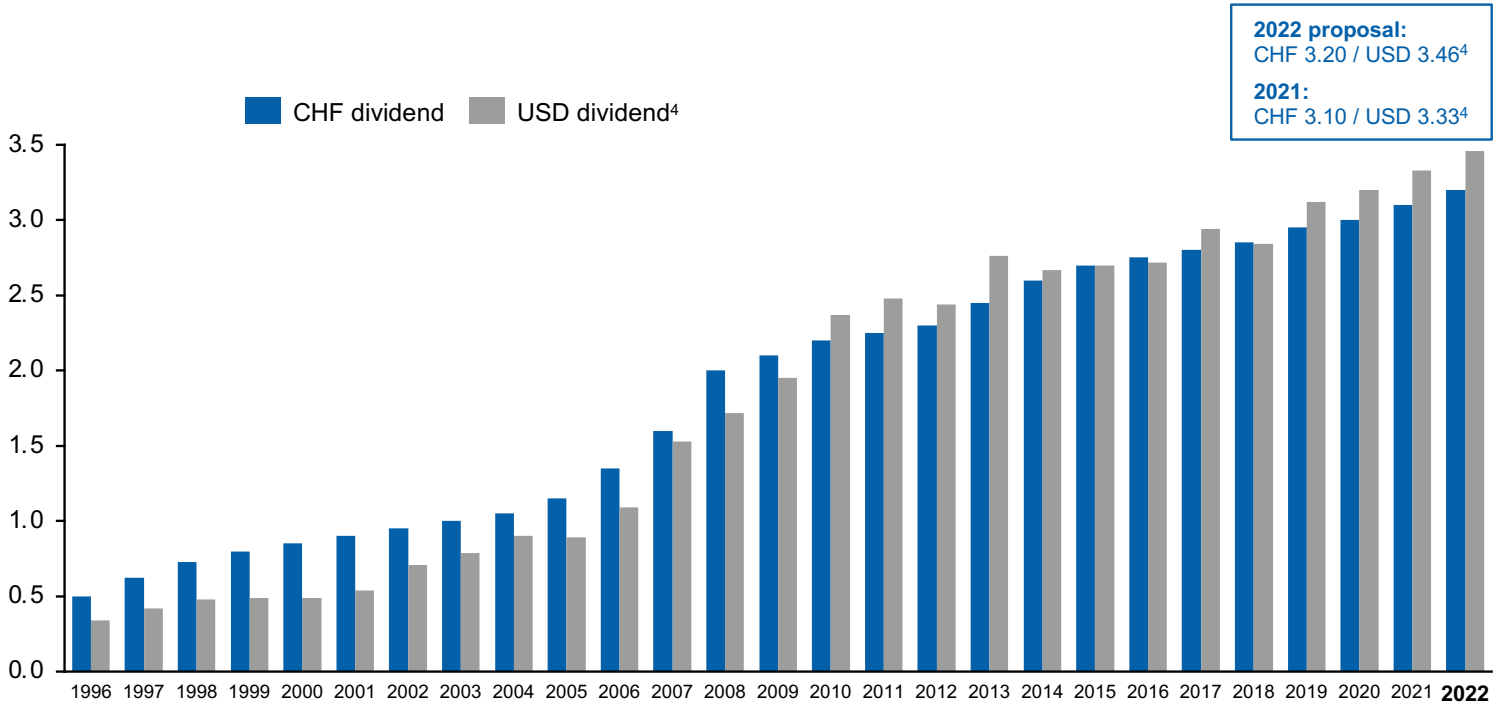
Dividend yield 3.8%[2]

Dividend growth 3.2%[3]

1996-2022 CAGR

CHF: 7.4%

USD: 9.3%[4]



1. Proposal to shareholders at the 2023 Annual General Meeting, taking place on March 7, 2023 2. Based on closing share price of CHF 83.59 at end of business year 2022 (December 30, 2022) 3. In CHF
4. Historical dividends per share converted at historical exchange rates at the dividend payment dates as per Bloomberg; for 2022, translated into US dollars at the FX rate of CHF/USD of 1.081, as of December 31, 2022

FINANCIAL PROFILE

Continuing core margin improvements for Group driven by IM

	Q4 2022				FY 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	3	14	36.4	3.5	4	8	36.9	1.3
Sandoz	0	-18	16.8	-3.8	4	-1	20.6	-1.1
Group	**3**	**15**	**31.8**	**3.5**	**4**	**8**	**33.0**	**1.3**

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.

FINANCIAL PROFILE

Sandoz returns to growth in 2022; planned spin-off on track for H2

Driven by Biopharma and ex-US sales

FY performance[1]

Sales USD 9.2bn (+4%)

Biopharma grew 9% and Retail 4%

Strong ex-US sales growth:

- EU: USD 4.9bn (+4%)
- RoW: USD 2.5bn (+9%)
- US: USD 1.8bn (-4%)

Core OpInc (-1%) impacted by inflationary pressures on input costs and higher SG&A

Key growth drivers for 2023 and beyond

Share gains across geographies

Two potential US biosimilar approvals expected in H2 2023

Launches, targeting USD 80bn originator sales (2030) with strong pipeline of 15+ biosimilar assets

Bolt-on BD&L and M&A

On track for planned spin-off in H2 2023

Final transaction requires Novartis AG BoD and shareholder approval

Carve-out financials to be provided ahead of CMD

Expected to be tax neutral to Novartis and the majority of our institutional shareholders

1. All growth rates in constant currencies (cc).

FINANCIAL PROFILE

Novartis excluding and including Sandoz 2023 full year guidance

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

Innovative Medicines (IM)	Sales expected to **grow low-to-mid single digit** Core OpInc expected to **grow mid-to-high single digit**
Novartis ex. Sandoz (IM + Corporate)	Sales expected to **grow low-to-mid single digit** Core OpInc expected to **grow mid-to-high single digit**
Novartis incl. Sandoz (IM + Sandoz + Corporate)[1]	Sales expected to **grow low-to-mid single digit** Core OpInc expected 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 2023
- We continue to expect that the planned Sandoz spin-off is completed in H2 2023

1. Novartis Group guidance, assuming Sandoz would remain within the Group for the entire FY 2023

FINANCIAL PROFILE

Sandoz 2023 and mid-term guidance

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

2023	Sales expected to **grow low-to-mid single digit** Core OpInc expected to **decline low double digit,** reflecting required stand-up investments to transition Sandoz to a separate company and continued inflationary pressures
Mid-term	Sales expected to **grow low-to-mid single digit CAGR** Core OpInc margin expected to **expand to mid 20s,** continuously progressing from the low 2023 base driven by continued Sales growth and operational efficiencies

Key assumptions:
We continue to expect that the planned Sandoz spin-off is completed in H2 2023

Note: after completion of planned Sandoz spin-off, Core OpInc guidance will be expressed in terms of core EBITDA.

FINANCIAL PROFILE

FY 2023 guidance on other financial KPIs

Barring unforeseen events; (in cc)

Group | Full year guidance

Core Net Financial Result	Expenses expected to be broadly in line vs. 2022
Core Tax Rate	Expected to be broadly in line vs. 2022

FINANCIAL PROFILE

Group Core OpInc to grow mid single digit as sales growth and SG&A savings partly offset by Gx erosion and Sandoz stand-up costs

2023 key drivers of core operating income (Group)

Vs. PY (cc) Illustrative

+ In-market growth drivers to continue growing strongly
+ Recent launches to further accelerate
+ China growth expected to accelerate benefiting from return to normal in H2
+ Simplified organizational model to deliver continued SG&A savings
+ Ongoing productivity programs

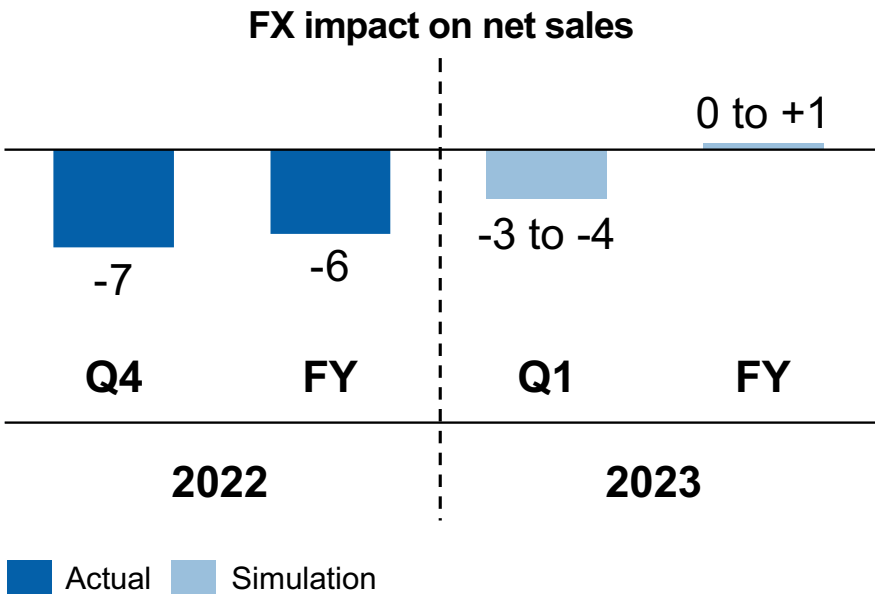
− Impact of inflation expected to continue in 2023
− Gx erosion expected to increase due to Gilenya® US and Lucentis® EU
− Stand-up investments to transition Sandoz to a standalone company

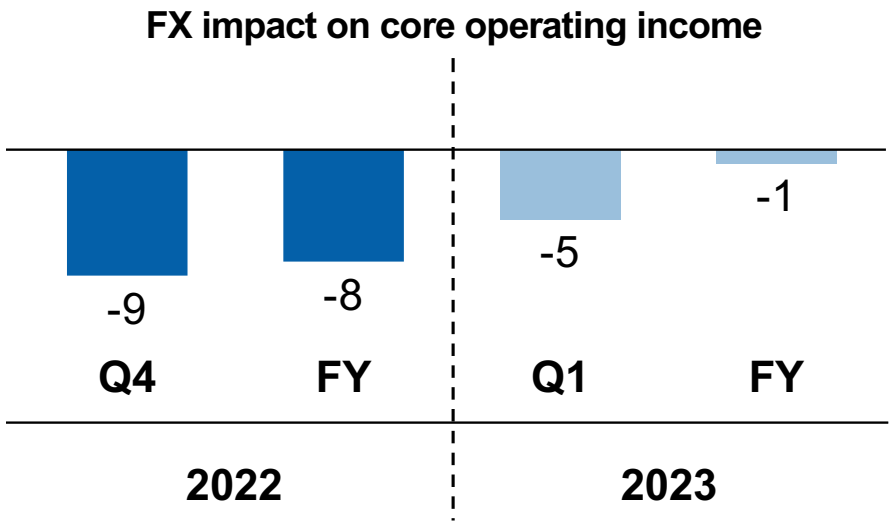
FINANCIAL PROFILE

Expected currency impact for 2023

Currency impact vs. PY

%pts, assuming late-January exchange rates prevail in 2023





Vas Narasimhan, M.D.

Chief Executive Officer

Creating impact through fulfilling unmet medical need by delivering innovative medicines to as many people possible

~290 million patients reached with Innovative Medicines and Novartis Global Health, and an additional **~453 million patients** reached with Sandoz

~150 pipeline projects further expanding patient reach

First gene, siRNA and radioligand therapies (at scale), fulfilling unmet medical need

~40 new drug approvals over the last 20 years, delivering innovative medicines

Recent innovation highlights:

Leqvio®	ASCVD
Scemblix®	CML
Pluvicto®	Prostate cancer
iptacopan	PNH and C3G

CONCLUSION

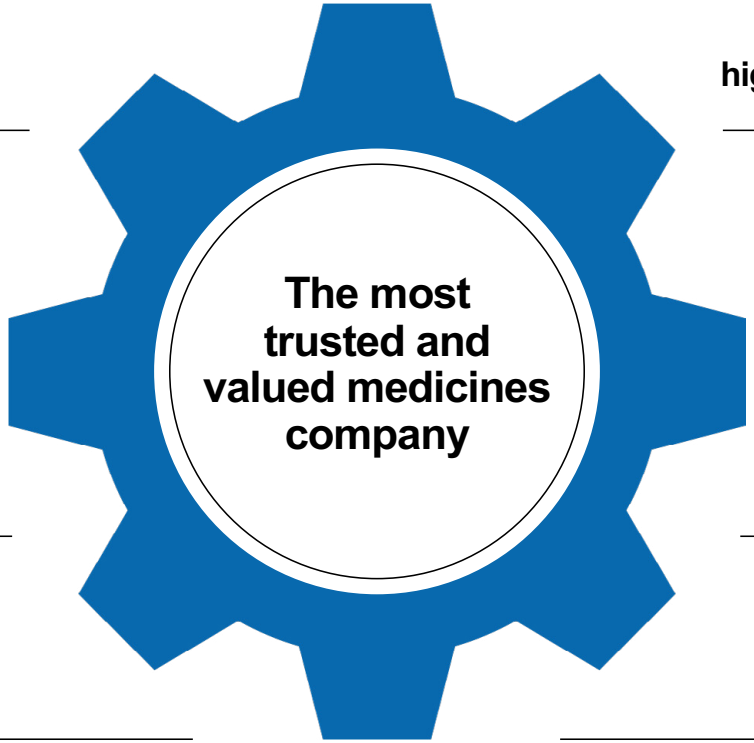
Novartis priorities for 2023 and beyond

1 Transforming to a **pure-play** IM company

2 **Focusing** on 5 core TAs, technology platforms and the US

3 Establishing **9 key brands** with multi-$bn potential

4 Improving **R&D productivity** (e.g. iptacopan, Pluvicto®)



5 Prioritizing pipeline in specific DAs to **high-value NMEs** across our 5 core TAs

6 Continuing to deliver **improved financials**

7 Continuing with **shareholder-focused capital allocation**

8 Strengthening foundations – **ESG/Human Capital**

Appendix

2022 events[1]

✓ Achieved to plan ✕ Not achieved to plan

NME Lead

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 ✓ /JPx[13])
	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) ✓[12]
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 x
	H2	Pluvicto® nmCRPC x[11]
	H2	YTB323 2L DLBCL[9] x
	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 updated. 10. FDA deferred action pending completion of required inspections. 11. Ph3 in nmCRPC shifting to Ph2, with FPFV in 2023. 12. Submission included also ankylosing spondylitis IV and non-radiographic axial SpA IV. 13. Base case approval moved to H1 2023.

2023 expected key events

		H1 2023	H2 2023
Regulatory decisions	**Cosentyx® HS**	EU	US
	Cosentyx® 2 ml AI	US	
	Cosentyx® IV		US
	Leqvio® Hypercholesterolemia		JP, China
Submissions	**Iptacopan PNH (US/EU/JP)**	US/EU	JP
	Pluvicto® mCRPC, pre-taxane (US)		US
Readouts	**Kisqali® HR+/HER2- BC (adj)**		NATALEE Ph3 FIR
	Iptacopan IgAN Ph3		APPLAUSE-IgAN Ph3
	Iptacopan C3G Ph3		Ph3 APPEAR-C3G Ph3
Ph3 starts	**Iptacopan in IC-MPGN**		Ph3
	Leqvio® CVRR primary prevention	Ph3	
	Ianalumab in immune thrombocytopenia	Ph3	
	Ianalumab in systemic lupus erythematosus	Ph3	

HS – hidradenitis suppurativa. PNH – paroxysmal nocturnalhemoglobinuria. mCRPC – metastatic castration-resistant prostate cancer. FIR – first interpretable results. IgAN – immunoglobulin A nephropathy. C3G – complement 3 Glomerulopathy. IC-MPGN – immune complex membranoproliferative glomerulonephritis.

Our pipeline projects at a glance

	Phase 1/2	Phase 3	Registration	Total
Innovative Medicines	**100**	**42**	**8**	**150**
Solid Tumors	24	15	3	42
Hematology	21	8	0	29
Immunology	23	7	4	34
Neuroscience	5	5	0	10
Cardiovascular	8	5	1	14
Others	19	2	0	21
Ophthalmology	*5*	*1*	*0*	*6*
Respiratory & Allergy	*3*	*0*	*0*	*3*
Global Health	*11*	*1*	*0*	*12*
Biosimilars[1]	**0**	**2**	**0**	**2**
Total	**100**	**44**	**8**	**152**

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	^{225}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
KFA115	KFA115	Novel immunomodulatory Agent	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	Hematological malignancy
JBH492	JBH492	-	Hematological malignancy
JEZ567	JEZ567	CD123 CAR-T	Acute myeloid leukemia
MAK683	MAK683	EED inhibitor	Cancers
MBG453	sabatolimab	TIM3 antagonist	Low risk myelodysplastic syndrome
MIK665	MIK665	MCL1 inhibitor	Hematological malignancies
PIT565	PIT565	-	B-cell malignancies
VAY736	ianalumab + ibrutinib	BAFF-R inhibitor	Hematological malignancy (combo)
VOB560	VOB560	-	Cancers
WVT078	WVT078	-	Multiple myeloma
YTB323	rapcabtagene autoleucel	CD19 CAR-T	Adult ALL

Cardiovascular

Code	Name	Mechanism	Indication(s)
XXB750	XXB750	NPR1 agonist	Cardiovascular diseases

Others

Code	Name	Mechanism	Indication(s)
Global Health			
EDI048	EDI048	CpPI(4)K inhibitor	Cryptosporidiosis
EYU688	EYU688	NS4B inhibitor	Dengue
KAF156	ganaplacide	Non-artemisinin plasmodium falciparum inhibitor	Malaria prophylaxis
INE963	INE963	-	Malaria, uncomplicated
Ophthalmology			
MHU650	MHU650	-	Diabetic eye diseases

Novartis pipeline in Phase 2

28 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
QUC398	QUC398	ADAMTS5 inhibitor	Osteoarthritis	
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
MIJ821	onfasprodil	NR2B negative allosteric modulator	Major depressive disorder with acute suicidal ideation or behavior

1. Gyroscope acquisition.

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 leukemia	
			Acute myeloid leukemia, maintenance	
PHE885	PHE885	BCMA cell therapy	4L multiple myeloma	
PKC412	Rydapt®	Multi-targeted kinase inhibitor	Acute myeloid leukemia, pediatrics	
YTB323	rapcabtagene autoleucel	CD19 CAR-T	1L high-risk large B-cell lymphoma	

Cardiovascular

Code	Name	Mechanism	Indication(s)	
CFZ533	iscalimab	CD40 inhibitor	Lupus nephritis	Type 1 diabetes mellitus
HSY244	HSY244	-	Atrial fibrillation	
LNP023	iptacopan	CFB inhibitor	Lupus nephritis	
MBL949	MBL949	-	Obesity related diseases	
TIN816	TIN816	ATP modulator	Acute kidney injury	
XXB750	XXB750	NPR1 agonist	Hypertension	

Others

Code	Name	Mechanism	Indication(s)	
Global Health				
KAE609	cipargamin	PfATP4 inhibitor	Malaria, severe	Malaria, uncomplicated
KAF156	ganaplacide	Non-artemisinin plasmodium falciparum inhibitor	Malaria, uncomplicated	
LXE408	LXE408	Proteasome inhibitor	Visceral leishmaniasis	
QMF149	Atectura®	Combo	Asthma, pediatrics	
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		SMURF1 inhibitor	Pulmonary arterial hypertension	
			Idiopathic pulmonary fibrosis	
Ophthalmology				
LKA651	LKA651	EPO inhibitor	Diabetic eye disease	
LNP023	iptacopan	CFB inhibitor	iAMD	
PPY988[1]	PPY988	Gene therapy - Complement factor I modulation	Geographic atrophy	

Novartis pipeline in Phase 3

8 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	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
			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 hemoglobinuria
			Atypical hemolytic uraemic syndrome
MBG453	sabatolimab	TIM3 antagonist	Myelodysplastic syndrome
VAY736	ianalumab	BAFF-R inhibitor	1L Immune Thrombocytopenia
			2L Immune Thrombocytopenia
			warm Autoimmune Hemolytic Anemia

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®	PGH-1 (artemisinin combination therapy)	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.

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
			Psoriatic arthritis (IV formulation)
			Axial SpA (IV formulation)
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

Cardiovascular | Immunology | Neuroscience | Solid tumors | Hematology | Non-core TA project

Lead

2023	2024	2025	≥2026			
iptacopan LNP023 PNH	**JDQ443** JDQ443 2/3L NSCLC (mono)	**NIS793** 1L Pancreatic cancer	**^{177}Lu-NeoB** AAA603 Multiple Solid Tumors	**iscalimab** CFZ533 Sjögren's syndrome	**MIJ821** Acute depression	**TNO155** Solid tumors
	remibrutinib LOU064 CSU	**pelacarsen** TQJ230 CVRR-Lp(a)	**gevokizumab** VPM087 1st line CRC	**ligelizumab** QGE031 Food allergy	**rapcabtagene autoleucel** YTB323 High-risk large B-cell lymphoma	**XXB750** Hypertension
	sabatolimab MBG453 HR-MDS		**ianalumab** VAY736 2L Immune Thrombocytopenia	**LNA043** Knee osteoarthritis		
			cipargamin KAE609 Malaria severe	libvatrep SAF312 COSP	LXE408 Visceral leishmaniasis	PPY988[1] Geographic atrophy
			ganaplacide KAF156 Malaria uncomplicated			

Supplementary

Pluvicto® AAA617 mCRPC, Pre-taxane	**iptacopan** LNP023 C3G		**ianalumab** VAY736 1L Immune Thrombocytopenia	**ianalumab** VAY736 Lupus Nephritis	**JDQ443** JDQ443 NSCLC (combo)	**sabatolimab** MBG453 Unfit AML
tislelizumab VDT482 1L Gastric Cancer	**iptacopan** LNP023 IgAN		**ianalumab** VAY736 wAIHA	**ianalumab** VAY736 SLE	**remibrutinib** LOU064 Multiple sclerosis	**tislelizumab** VDT482 Adj/Neo adj NSCLC
tislelizumab VDT482 1L ESCC	**Pluvicto®** AAA617 mHSPC		**ianalumab** VAY736 AIH	**iptacopan** LNP023 aHUS	**remibrutinib** LOU064 Sjögren's syndrome	**tislelizumab** VDT482 1L Urothelial Cell Carcinoma
tislelizumab VDT482 1L Hepatocellular Carcinoma	**tislelizumab** VDT482 1L Small Cell Lung Cancer		**ianalumab** VAY736 Sjögren's syndrome			
tislelizumab VDT482 1L Nasopharyngeal cancer	**tislelizumab** VDT482 Localized ESCC		cipargamin KAE609 Malaria uncomplicated			

1. Gyroscope acquisition.

Novartis submission schedule

Supplementary indications for existing brands

Cardiovascular | Immunology | Neuroscience | Solid tumors | Hematology | Non-core TA project

Existing brands

2023	2024	2025	≥2026
Kisqali® ribociclib, LEE011 HR+/HER2- BC (adj)	**Jakavi®** ruxolitinib, INC424 Pediatrics Acute GVHD	**Cosentyx®** secukinumab, AIN457 GCA	**Aimovig®** erenumab, AMG334 Pediatric Migraine
Lutathera® ^{177}Lu-oxodotreotide[1] GEP-NET 1L G3	**Jakavi®** ruxolitinib, INC424 Pediatrics Chronic GVHD	**Leqvio®** KJX839 Ped Hyperlipidemia	**Cosentyx®** secukinumab, AIN457 Lupus Nephritis
Piqray® alpelisib, BYL719 Ovarian cancer		**Scemblix®** ABL001 CML 1L	
Xolair® omalizumab, IGE025 Food allergy		**Zolgensma®** AVXS-101 OAV101 SMA IT	
denosumab BioS GP2411 anti RANKL mAb	**Adakveo** SEG101 Sickle cell disease, pediatrics	**Beovu®** brolucizumab, RTH258 Diabetic retinopathy	**Atectura®** indacaterol + mometasone, QMF149 Asthma, pediatrics
	aflibercept BioS SOK583 Neovascular age-related macular degeneration	**Promacta®** eltrombopag, ETB115 Radiation sickness syndrome	
	Coartem® artemether + lumefantrine, COA566 Malaria uncompl., formula for <5kg		

≥2026		
Kesimpta® [2] ofatumumab Multiple sclerosis, pediatrics	**Mayzent®** [2] siponimod, BAF312 Multiple sclerosis, pediatrics	**Scemblix®** ABL001 CML, 2L, pediatrics
Leqvio® KJX839 CVRR-LDLC	**Rydapt®** midostaurin, PKC412 Acute myeloid leukemia, pediatrics	

1. ^{177}Lu-dotatate in US. 2. 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

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
Readout 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

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
Readout Milestone(s)	2025
Publication	Wong et al 2021 Nephrology, Dialysis and Transplantation Vol. 36, Suppl. 1: eGFR trajectory

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
Readout 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
Readout 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)
Readout 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)
Readout Milestone(s)	2025
Publication	Design publication (O-16/-13) in Eur. J. Prev. Cardiol. Vol. 29, Feb. 2022 (actual) Presentation at EAS May-2022 on O-13/-16 study design (actual)

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)
Readout Milestone(s)	2025
Publication	Design publication (O-16/-13) in Eur. J. Prev. Cardiol. Vol. 29, Feb. 2022 (actual) Presentation at EAS May-2022 on O-13/-16 study design (actual)

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)
Readout Milestone(s)	2027
Publication	TBD

pelacarsen - Antisense oligonucleotide (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
Readout Milestone(s)	2025
Publication	TBD

XXB750 - NPR1 agonist

NCT05562934 (CXXB750B12201)

Indication	Hypertension
Phase	Phase 2b
Patients	170
Primary Outcome Measures	Change from baseline in mean 24hr ambulatory systolic blood pressure at week 12
Arms Intervention	Arm 1 experimental: Dose 1 Arm 2 experimental: Dose 2 Arm 3 experimental: Dose 3 Arm 4 experimental: Dose 4 Arm 5 placebo comparator
Target Patients	Resistant Hypertension Patients
Readout Milestone(s)	2024
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)
Readout Milestone(s)	2025
Publication	TBD

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)
Readout Milestone(s)	Primary 2025 Final 2026
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT03217422 AMBER (CVAY736B2201)

Indication	Autoimmune hepatitis
Phase	Phase 2
Patients	65
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
Readout Milestone(s)	2024
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: Experimental - ianalumab s.c. q12w in addition to SoC Arm 3: Placebo comparator - Placebo s.c. q4w in addition to SoC
Target Patients	Patients with active Lupus Nephritis
Readout 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
Readout 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
Readout 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
Readout 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
Readout 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
Readout 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
Readout 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
Readout Milestone(s)	2024
Publication	TBD

Neuroscience

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.
Readout 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
Readout 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
Readout 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
Readout 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
Readout 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)
Readout Milestone(s)	2024
Publication	TBD

Oncology

ianalumab - BAFF-R inhibitor

NCT05653349 VAYHIT1 (CVAY736I12301)

Indication	1L Immune Thrombocytopenia
Phase	Phase 3
Patients	225
Primary Outcome Measures	Time from randomization to treatment failure (TTF)
Arms Intervention	Arm 1: Experimental: Ianalumab Lower dose administered intravenously with corticosteroids oral or parentally (if clinically justified) Arm 2: Ianalumab Higher dose administered intravenously with corticosteroids oral or parentally (if clinically justified) Arm 3: Placebo Comparator administered intravenously with corticosteroids oral or parentally (if clinically justified)
Target Patients	Adult patients with primary ITP
Readout Milestone(s)	2025
Publication	TBD

NCT05653219 VAYHIT2 (CVAY736Q12301)

Indication	2L Immune Thrombocytopenia
Phase	Phase 3
Patients	150
Primary Outcome Measures	Time from randomization to treatment failure (TTF)
Arms Intervention	Arm 1: Experimental: eltrombopag and ianalumab lower dose Arm 2: Experimental: eltrombopag and ianalumab higher dose Arm 3: eltrombopag and placebo
Target Patients	Primary ITP patients who failed steroids
Readout Milestone(s)	2025
Publication	TBD

ianalumab - BAFF-R inhibitor

NCT05648968 VAYHIA (CVAY736O12301)

Indication	Warm autoimmune hemolytic anemia
Phase	Phase 3
Patients	90
Primary Outcome Measures	Binary variable indicating whether a patient achieves a durable response Durable response: hemoglobin level ≥10 g/dL and ≥2 g/dL increase from baseline, for a period of at least eight consecutive weeks between W9 and W25, in the absence of rescue medication or prohibited treatment
Arms Intervention	Arm 1: experimental Ianalumab low dose (intravenously) Arm 2: experimental Ianalumab high dose (intravenously) Arm 3: placebo Comparatorn (intravenously)
Target Patients	Previously treated patients with warm Autoimmune Hemolytic Anemia
Readout Milestone(s)	2026
Publication	TBD

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)
Readout Milestone(s)	2025
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
Readout 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
Readout 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.
Readout 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
Readout 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
Readout Milestone(s)	Primary: 2024
Publication	NA

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
Readout 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% ^{177}Lu-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
Readout Milestone(s)	Primary Analysis: 2022 (actual) 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: ^{177}Lu-PSMA-617 Participant will receive 7.4 GBq (+/- 10%) ^{177}Lu-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)
Readout Milestone(s)	Primary Analysis: 2024
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)
Readout Milestone(s)	2026
Publication	TBD

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
Readout 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)
Readout 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
Readout Milestone(s)	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
Readout Milestone(s)	2024
Publication	TBD

Other

Ophthalmology

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
Readout 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.
Readout Milestone(s)	2023
Publication	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
Readout 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
Readout Milestone(s)	2024
Publication	TBD

Coartem® - PGH-1 (artemisinin combination therapy)

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
Readout Milestone(s)	Primary outcome measure: 2023
Publication	TBD

ganaplacide - Non-artemisinin plasmodium falciparum inhibitor

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
Readout 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
Readout Milestone(s)	2023
Publication	tbd

Abbreviations

AI	Auto-injector
AIH	Autoimmune hepatitis
aHUS	atypical Hemolytic Uremic Syndrome
ALL	Acute lymphoblastic leukemia
ALS	Amyotrophic lateral sclerosis
AML	Acute myeloid leukemia
BC	Breast cancer
C3G	C3 glomerulopathy
CART	Chimeric androgen receptor T
CLL	Chronic lymphocytic leukemia
CML	Chronic myeloid leukemia
CRC	Colorectal cancer
COPD	Chronic obstructive pulmonary disease
COSP	Chronic ocular surface pain
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
GRPR	Gastrin releasing peptide receptor
HCC	Hepatocellular carcinoma
HD	Huntington's disease
HR LBCL	High risk large B-cell lymphoma
IA	Interim analysis
iAMD	Intermediate age-related macular degeneration
IC-MPGN	Immune complex membranoproliferative glomerulonephritis
IgAN	IgA nephropathy
IPF	Idiopathic pulmonary fibrosis
ITP	Immune thrombocytopenia
LBCL	Large B-cell lymphoma
LN	Lupus nephritis
mCRPC	Metastatic castration-resistant prostate cancer
MDS	Myelodysplastic syndrome
mHSPC	Metastatic hormone sensitive prostate cancer
mPDAC	Metastatic pancreatic ductal adenocarcinoma
MS	Multiple sclerosis
NASH	Non-alcoholic steatohepatitis
nmCRPC	Non-metastatic castration-resistant prostate cancer
NPR1	Natriuretic peptide receptor 1
nr-axSpA	Non-radiographic axial spondyloarthritis
NSAI	Non-steroidal aromatase inhibitor
NSCLC	Non-small cell lung cancer
OS	Overall survival
PFS	Prefilled syringe
PNH	Paroxysmal nocturnal haemoglobinuria
PsA	Psoriatic arthritis
rHR	Resistant hypertension
rMS	Relapsing multiple sclerosis
rPFS	Radiographic progression free survival
SLE	Systemic lupus erythematosus
SMA Type 1	Spinal muscular atrophy (IV formulation)
SMA Type 2/3	Spinal muscular atrophy (IT formulation)
SpA	Spondyloarthritis
T1DM	Type 1 Diabetes mellitus
wAIHA	Warm autoimmune hemolytic anemia