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Q2 2025 Results

Investor presentation
July 17, 2025

NOVARTIS | Reimagining Medicine

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This presentation includes non-IFRS financial measures, including Constant currencies (cc), core results and free cash flow. An explanation of non-IFRS measures can be found on page 40 of the Novartis Second Quarter and Half Year 2025 Condensed Interim Financial Report.

Content
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Company overview

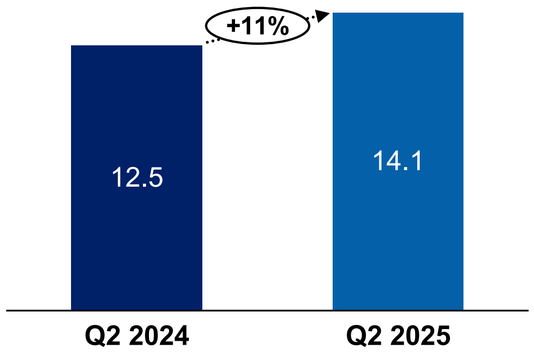
Vas Narasimhan, M.D.
Chief Executive Officer

NOVARTIS | Reimagining Medicine

Novartis delivered double-digit sales growth and core margin expansion in Q2, supporting an upgrade to FY 2025 bottom-line guidance

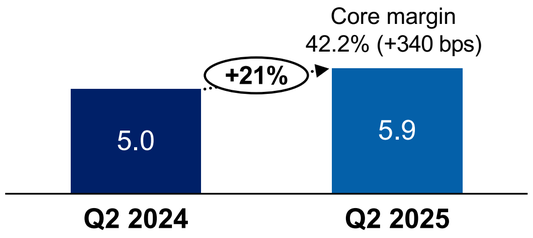
Sales

USD bn, % cc[1]



Core[1] operating income

USD bn, % cc



Innovation highlights

Pluvicto® Phase III positive readout in mHSPC

Vanrafia® FDA accelerated approval for IgAN

OAV101 IT US and EU submissions for SMA

Votoplam Phase II positive readout in Huntington's

Remibrutinib Phase II positive readout in food allergy

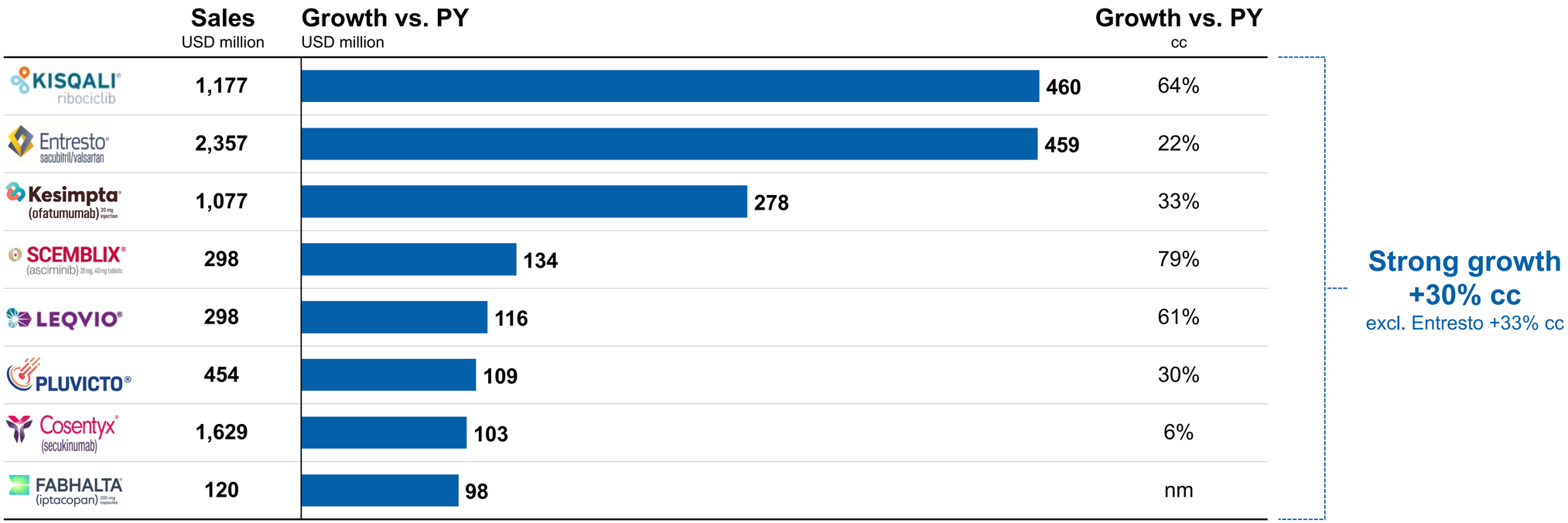
YTB323 data in SLE presented at EULAR

FY 2025 core operating income guidance upgraded to low-teens growth (from low double-digit)[2]

1. Constant currencies (cc) and core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.
2. Please see detailed guidance assumptions on slide 23.

Priority brands continued to drive robust growth, demonstrating the replacement power in our portfolio

Q2 sales



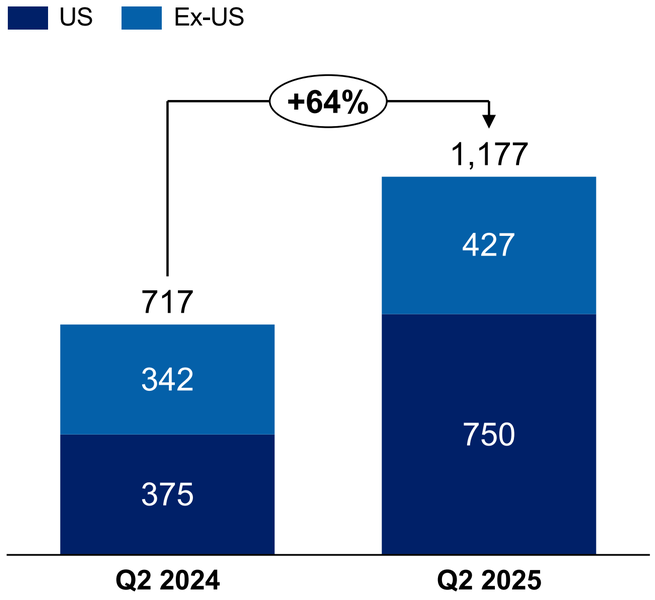
Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Kisqali® grew +64% cc in Q2, achieving TRx leadership in mBC and building momentum in eBC

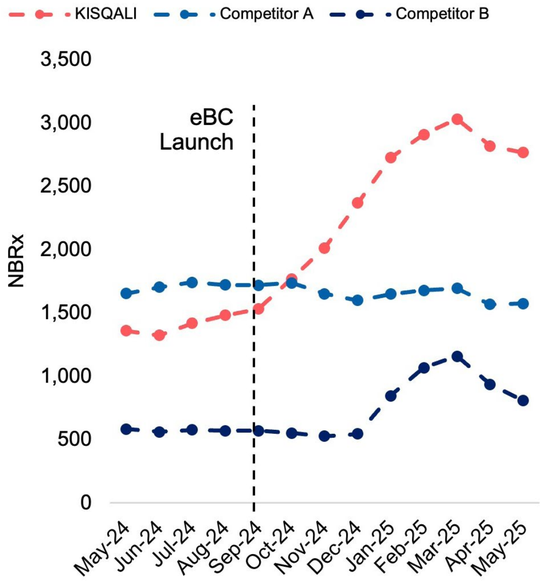
KISQALI®

Sales evolution

USD m, % cc



US Total Brand NBRx (R3M)[1]



NBRx volume impacted in Q1 by reverification

US: +100% in Q2

- mBC leadership in both NBRx (50% share) and TRx (37% share)[2]
- eBC NBRx 61% share[2], with leadership in overlapping and exclusive populations

Ex-US: +25% cc in Q2

- mBC leader in both NBRx (50%)[3] and TRx (38%)[3]
- eBC now approved in EU, China + 18 countries
- First launch markets following US trajectory with Germany eBC NBRx share at 71%[4]

Strong guideline support

- Category 1 preferred NCCN Guidelines
- Only CDK4/6 with highest ESMO scores in eBC and mBC

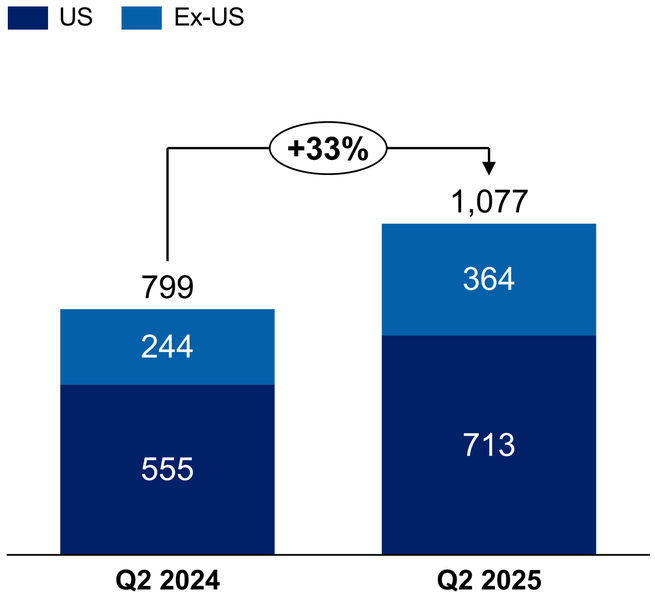
See page 77 for references (footnotes 1-4). Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Kesimpta® grew +33% cc in Q2, fueled by continued strong demand growth

Kesimpta

Sales evolution

USD m, % cc



US: +28% in Q2

- Continued TRx growth +23% vs. PY, gaining +2.3pts market share[1]
- Access improvements translating to fewer bridge and more direct-to-paid starts
- Opportunity remains: ~50% of patients still on low-efficacy therapies[2]

Ex-US: +45% cc in Q2

- Leading NBRx share in patients in 8/10 major markets[3]
- Opportunity remains: ~70% of DMT-treated patients in Europe not treated with a B-cell therapy[4]

Only self-administered B-cell treatment option

- Intentionally designed for self-administration
- One minute, once a month, at home or on the go, no pre-medications[5]

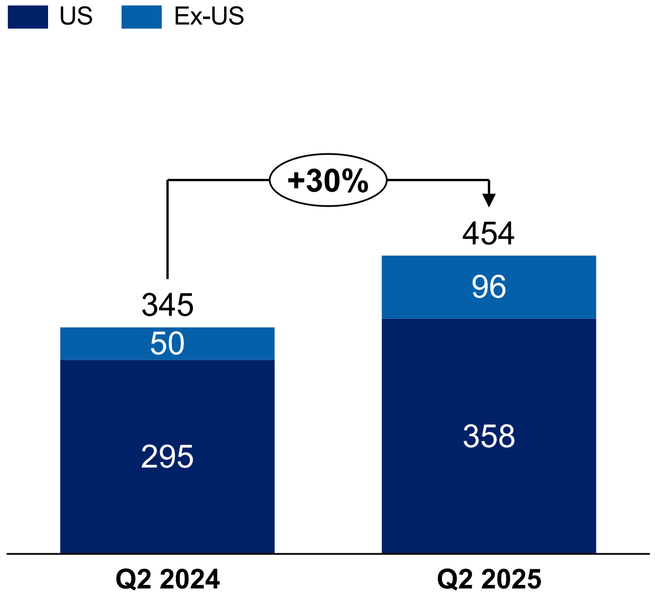
See page 77 for references (footnotes 1-5). Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Pluvicto® grew +30% cc in Q2, with promising uptake since pre-taxane indication approval in US

PLUVICTO®

Sales evolution

USD m, % cc



US pre-taxane launch off to a strong start

- Significant QoQ growth in new patient starts (~40%)[1] and sales (~25%) in Q2
- Record high new patient starts in June following typical 4–7-week treatment lag-time

Key success factors in place for continued growth in US

- Strong uptake in community setting (~60% NBRx QoQ growth; 58% of TRx in Q2)[2]
- ~9/10 patients estimated to be within 30 miles of a treatment site (>670 sites, ~40% growth vs. PY)
- >50% of PSMAfore patients treated by key HCPs who have used Pluvicto in VISION setting

Ex-US growth continues in VISION setting

- Growth driven by Europe and expanded access

See page 77 for references (footnotes 1-2). Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Positive Phase 3 PSMAddition study for Pluvicto® paves the way for potential further expansion in mHSPC

PLUVICTO®

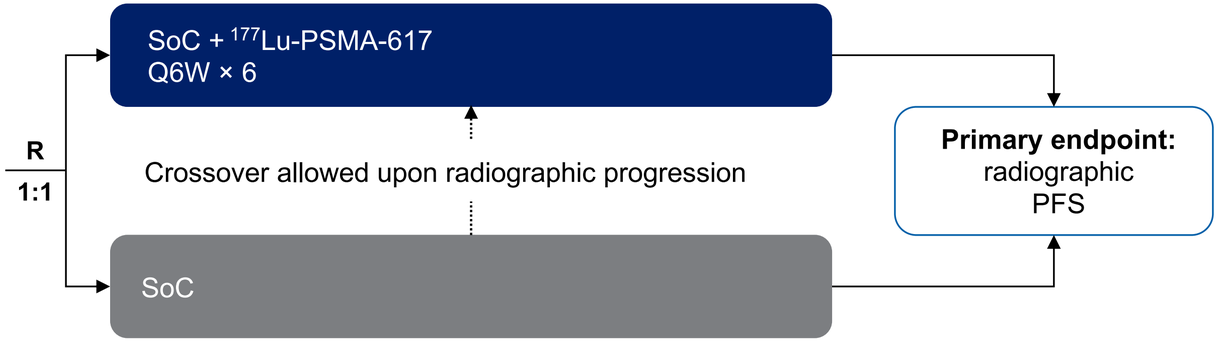
Primary endpoint met

- Statistically significant and clinically meaningful benefit on radiographic progression-free survival (rPFS)
- Positive trend in overall survival (OS)
- Data will be presented at an upcoming medical congress

Significant opportunity

- US incidence of mHSPC (~42.5k) comparable to mCRPC (~44k)
- Expanding breadth in community oncology and urology for PSMAfore launch will also serve PSMAddition

Study design[1]



Patient population[2]	Untreated or minimally treated mHSPC	Appropriate SoC (ADT and ARPI)	PSMA-positive on [68Ga]Ga-PSMA-11 PET/CT

Based on FDA feedback, submission planned in H2 2025

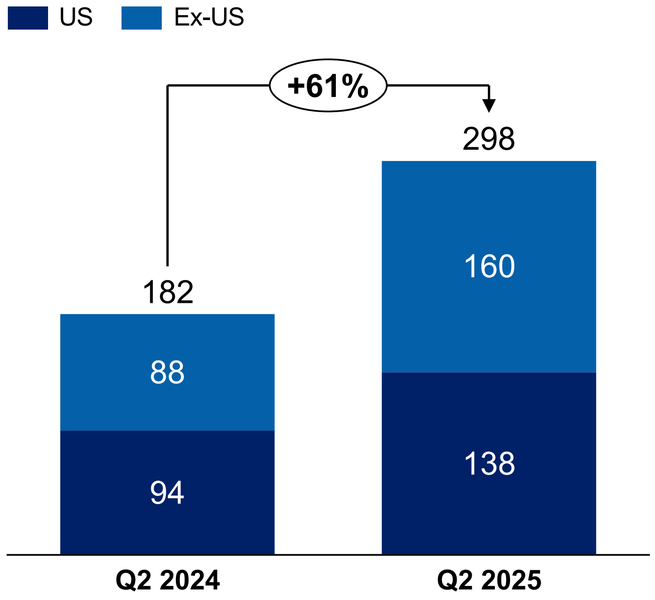
See page 77 for references (footnotes 1-2).

Leqvio® grew +61% cc in Q2, on track for blockbuster status in 2025

LEQVIO® (inclisiran)

Sales evolution

USD m, % cc



US: +47%, outpacing advanced lipid-lowering market[1,2]

- MOTRx +56% vs. PY (market +35%)
- Increasing depth in priority health systems, +34% vs. PY[3]
- Continued focus on post-event patients, leveraging V-INCEPTION results and updated 2025 ACC/AHA guidelines for ACS[4]

Ex-US: +74% cc, driven by sustained growth in all markets

- Continued out-of-pocket market expansion in China

Continuing to build Leqvio evidence base

- Pediatric submission underway building on the successful completion of ORION-13 and ORION-16
- Global V-MONO trial (superiority of Leqvio vs both placebo and ezetimibe) presented at EAS
- V-INCEPTION (inclisiran implementation strategy post-ACS) presented at NLA Scientific Sessions

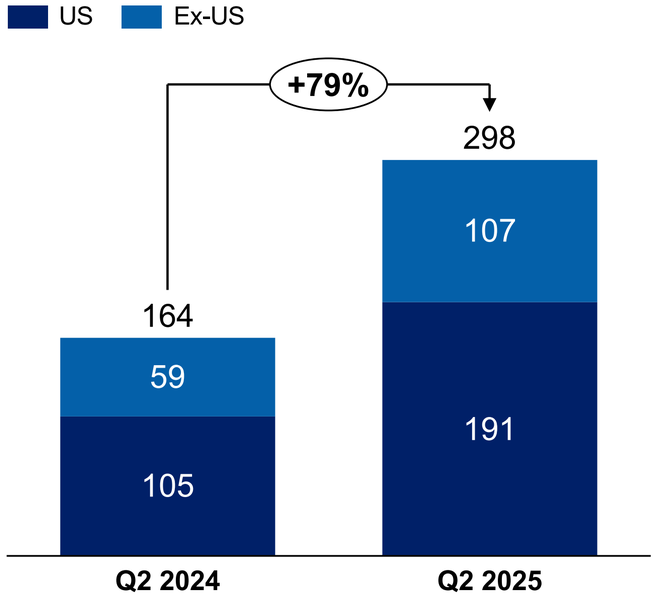
See page 78 for references (footnotes 1-4). Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY. Novartis obtained global rights to develop, manufacture, and commercialize Leqvio under license/collaboration agreement with Alnylam Pharmaceuticals.

Scemblix® grew +79% cc in Q2, on track for blockbuster status in 2025 with strong momentum in early lines and global leadership in 3L+ CML

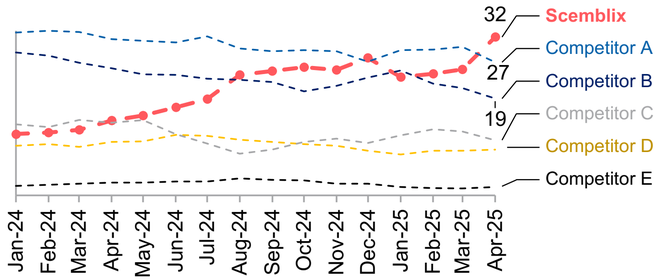
SCEMBLIX® (asciminib) 20 mg, 40 mg tablets

Sales evolution

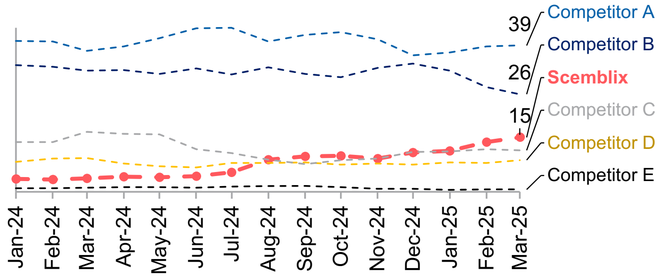
USD m, % cc



US All LoT NBRx Share (R3M)[1]



US 1L NBRx Share (R3M)[1]



US: Achieved NBRx leadership across all lines of therapy

- 1L NBRx share 15% (Mar ’25 R3M), vs.10% in Jan[1]
- NBRx leader in 2L and 3L+ with 44% and 55% share, respectively[1]

Ex-US: 3L+ leadership with increasing early line approvals

- 48% total share in key markets[2]
- Early lines indication approved in 20 countries (including China and Japan)

Strong clinical data

- ASC4START 1L and ASC2ESCALATE 2L at ASCO and EHA

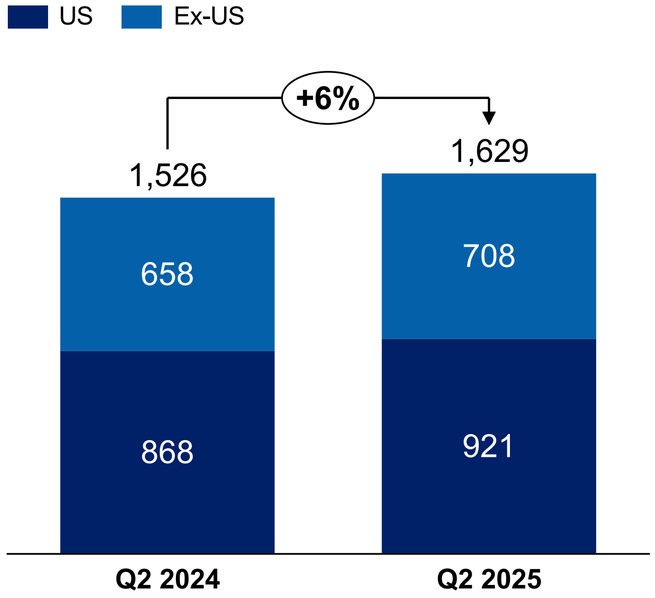
See page 78 for references (footnotes 1-2). Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Cosentyx® growth moderated to +6% cc in Q2; expect mid-single digit growth in FY 2025

Cosentyx®

Sales evolution

USD m, % cc



Continued solid demand for launches in US (HS, IV)

- HS: Continuing to grow the market, with 70% of business from naïve patients; leading NBRx (52% share in naïve, 48% overall)[1]
- IV: Continued steady growth in IV formulation (+17% vials growth QoQ)[2]

Competitive in core indications globally (PsO, SpA)

- US: #1 IL17 prescribed across indications, supported by strong access
- Ex-US: Leading originator biologic in EU[3] and China[4]

Facing geography-specific short-term headwinds

- US: Higher RDs (340B, Medicare Part D redesign impact), increased competition, strong launch performance in PY
- Ex-US: Pricing impacts from indication expansion, market slowdown in China[4]

Remain confident in USD 8bn+ peak sales potential

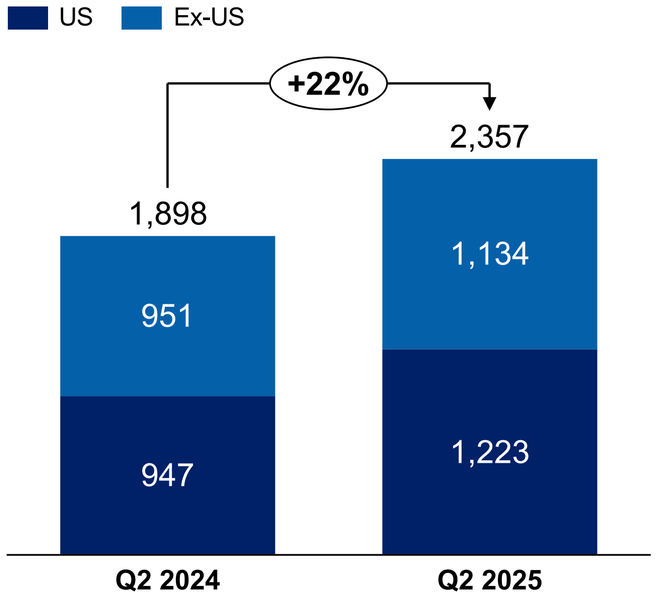
See page 78 for references (footnotes 1-4). Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY. SpA refers to the Cosentyx indications in psoriatic arthritis (PsA), non-radiographic axial spondyloarthritis (nr-axSpA), and ankylosing spondylitis (AS).

Entresto® grew +22% cc in Q2, as consistent performance continued

Entresto®

Sales evolution

USD m, % growth



Fully met Entresto® US mid-2025 financial planning assumption

- IP and regulatory litigation continues against one generic, who is currently enjoined from any launch
- Any later launch prior to the final outcome of these litigations may be at risk of later litigation developments[1]

Expect continued growth ex-US

- Strong guideline position[2] (US/EU)
- Balanced geographic sales[3]: US ~50%, Europe ~20%, China ~10%, Japan ~5%
- Ex-US: RDP to Nov 2026[4] in EU, Jun 2030 in Japan, with possible additional protection

See page 78 for references (footnotes 1-4). Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Renal portfolio continues to advance with multiple ongoing launches and new long-term data for zigakibart

Fabhalta

- **IgAN** growing steadily in US, with continued high persistency and compliance
- Initial use aligned to positioning for patients with persistent proteinuria and **glomerular inflammation**[1]
- **C3G** positive early launch signals in US, reflecting high unmet need; now approved in >30 countries including Japan

Vanrafia[2]

- Strong HCP feedback on clinical data, **no REMS** and **seamless oral add-on** to supportive care[3]
- **Exceeding early targets** for patient enrollments and new writers in US
- Early access wins within 3 months; on track for **broad commercial coverage**[4]

Zigakibart

- 100-week data from ongoing Phase I/II trial (n=40) represents **longest duration of treatment reported for an anti-APRIL** to date
- Clinically meaningful proteinuria reduction of **60.4%,** sustained eGFR stabilization, no AEs leading to treatment discontinuation/death
- BEYOND Ph3 study on track with recruitment nearly completed; readout expected in **H1 2026**

See page 79 for references (footnotes 1-4).

Remibrutinib demonstrated a clinically meaningful and statistically significant benefit in Phase II food allergy study

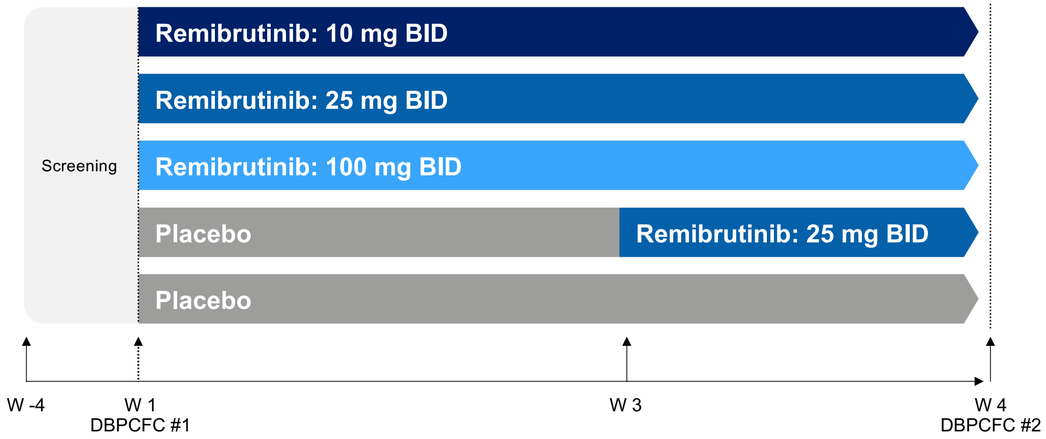
Primary endpoint met

- Primary endpoint: Tolerating ≥ 600 mg of peanut protein at Week 4
- Safety results were consistent with the overall safety profile of remibrutinib

Significant opportunity for an effective oral option

- Food allergy has a global prevalence of ~3-8%[1,2]
- Allergen avoidance is burdensome and unreliable
- Current treatment options are limited
- Remibrutinib has the potential to become the first oral allergen-agnostic treatment with fast onset of action

Study design



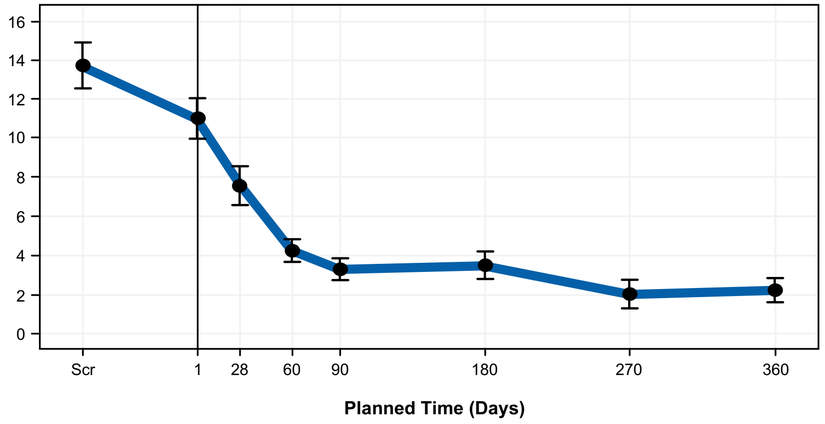
Population: ~70 adult patients with peanut allergy assessed for desensitization via a double-blind placebo-controlled food challenge (DBPCFC)

Next steps: Phase II data will be presented at a medical congress in H1 2026; Phase III planning underway

See page 79 for references (footnotes 1-2).

YTB323[1]: Interim Phase I/II data[2] indicate sustained clinical improvement in severe refractory Systemic Lupus Erythematosus (SLE)

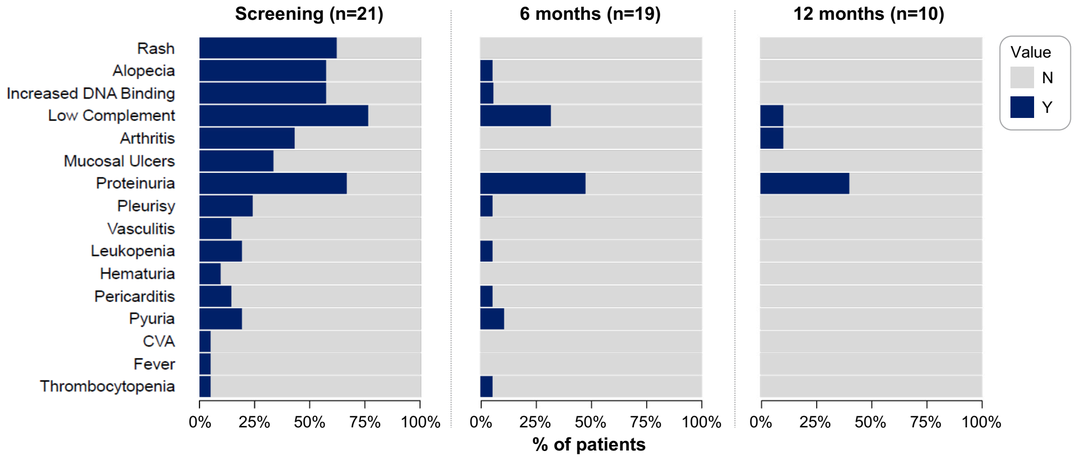
SLEDAI-2K total score over time, mean (SE)[3]



Early and sustained improvement of overall disease activity in patients with srSLE (n=21) with up to 12 months follow-up

Safety in line with CAR-T therapy experience

Distribution of SLEDAI−2K items[4] at select timepoints



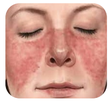
Marked improvement across SLEDAI items except for persistent proteinuria in some patients, which may represent irreversible kidney damage[5]

See page 79 for references (footnotes 1-5).

Advancing a broad development program for YTB323 in autoimmune diseases, including four pivotal studies

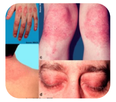
Rheumatology

srSLE/LN

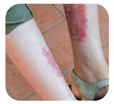


SSc

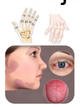
IIM



AAV

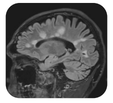


RA/SjD

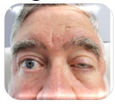


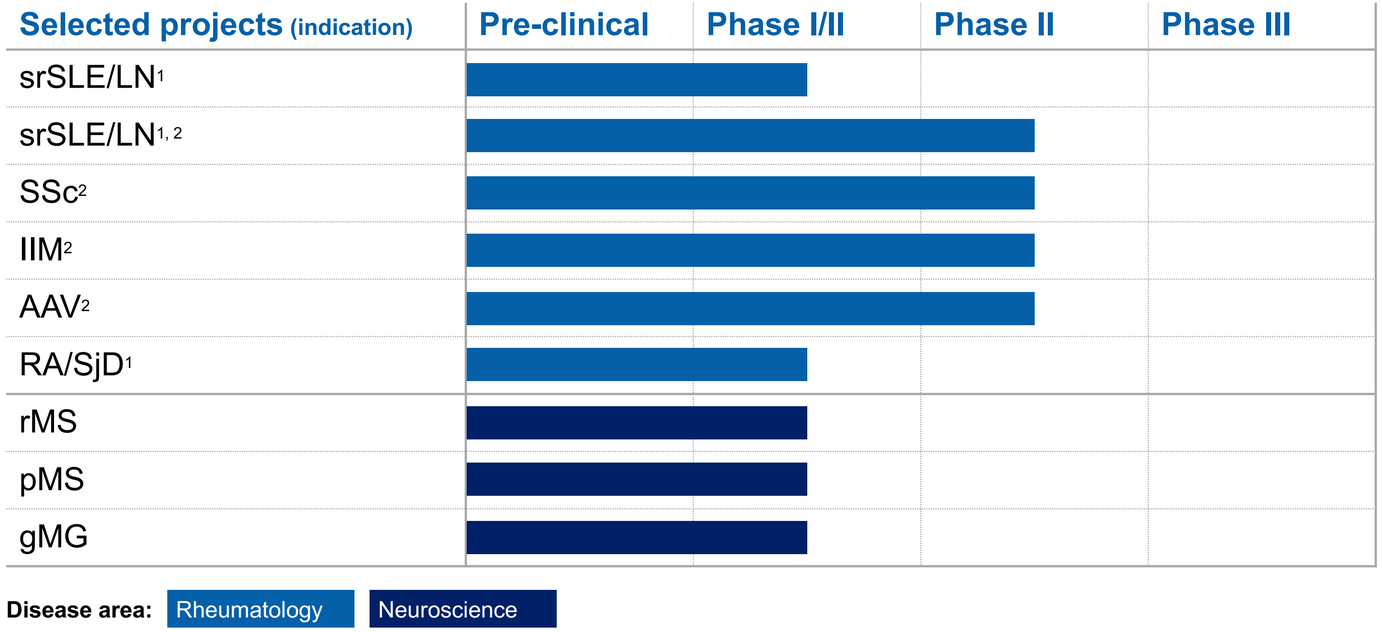
Neuroscience

MS



gMG





Next steps: Recruitment ongoing for 4 pivotal Phase II studies, first readout ≥2027

See page 79 for references (footnotes 1-2).

Key innovation milestones in 2025

2025 selected key events (expected)		H1 2025	H2 2025	Status as of end Q2
Regulatory decisions	**Atrasentan IgAN**	US		US approval (Q2)
	Fabhalta® (iptacopan) C3G	US, JP	EU	US, EU approvals in Q1; China, JP approvals in Q2
	Pluvicto® mCRPC, pre-taxane	US		US approval (Q1)
	Scemblix® 1L CML		JP	JP, China approvals (Q2)
Submissions	**Remibrutinib CSU**	US, EU, CN		US, EU and China submissions (Q1), China priority review granted
	Zolgensma® SMA IT	US, EU	JP	US, EU submissions (Q2)
	Scemblix® CML 1L	EU		EU submission (Q1)
	Pluvicto® mHSPC		US	
	Cosentyx® GCA		US, EU	See below
Readouts	**Cosentyx® GCA**	Ph3 (GCAPTAIN)		Did not meet primary endpoint (Q2); Safety consistent with known safety profile of Cosentyx®
	Cosentyx® PMR		Ph3 (REPLENISH)	
	Ianalumab SjD		Ph3s (NEPTUNUS-1 and -2)	
	Ianalumab 2L ITP		Ph3 (VAYHIT2)	
	Pluvicto® mHSPC		Ph3 (PSMAddition)	Met its primary endpoint (Q2)
	Remibrutinib FA		Ph2	Met its primary endpoint (Q2)
	Ianalumab HS	Ph2		Predefined efficacy thresholds for the PoC not achieved
	Votoplam (PTC518) HD[1]	Ph2 (PIVOT-HD)		Met its primary endpoint (Q2)
Key study starts	**Remibrutinib HS**	Ph3		Ph3 trials RECHARGE-1 and -2 started (Q1)
	Remibrutinib gMG	Ph3		Ph3 trial RELIEVE started (Q1)
	Ac-PSMA-617 PC	Ph3		Ph3 trial ActFIRST started (Q2)
	YTB323 AAV	Ph2		Ph2 trial started (Q1)
	JSB462 (AR degrader) PC		Ph2	Ph2 trials started (Q2)
	GIA632 (IL-15 mAb)		Ph2	
	QCZ484 HTN		Ph2	Ph2 trial started (Q1)
	VHB937 (TREM2) AD		Ph2	

1. Ongoing study shown is sponsored by PTC Therapeutics. Novartis has obtained global rights to develop, manufacture, and commercialize votoplam under License & Collaboration agreement with PTC Therapeutics.

Financial review and 2025 guidance

Harry Kirsch
Chief Financial Officer

NOVARTIS | Reimagining Medicine

Q2 net sales grew +11% cc[1], with strong core[1] margin expansion

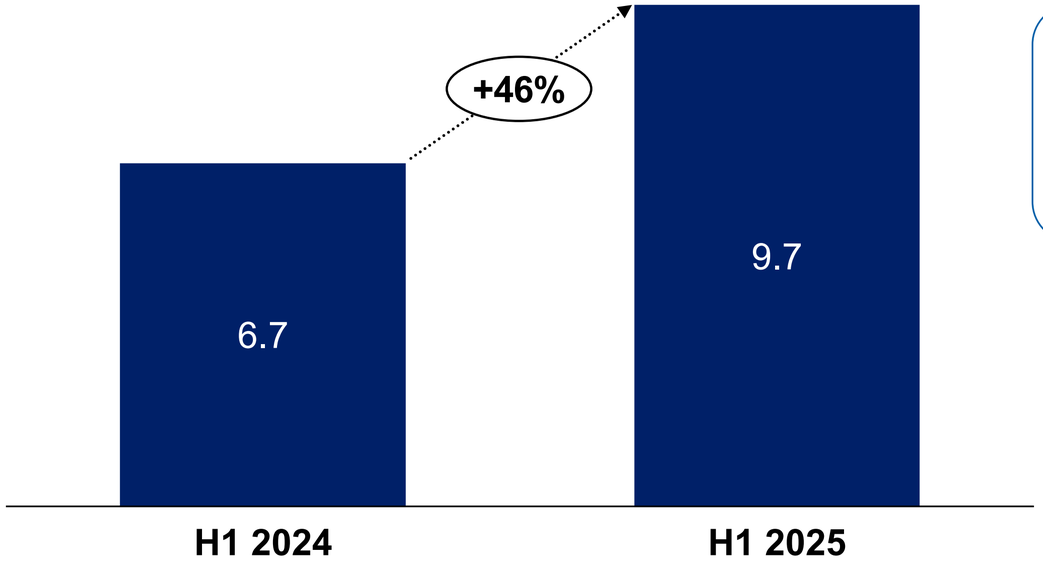
Key figures[1] USD million	Q2 2024	**Q2 2025**	**Change vs. PY** % USD	% cc	H1 2024	**H1 2025**	**Change vs. PY** % USD	% cc
Total net sales	12,512	14,054	12	11	24,341	27,287	12	13
Core operating income	4,953	5,925	20	21	9,490	11,500	21	24
Core margin	*39.6%*	*42.2%*	*+2.6%pts*	*+3.4%pts*	*39.0%*	*42.1%*	*+3.1%pts*	*+3.7%pts*
Operating income	4,014	4,864	21	25	7,387	9,527	29	33
Net income	3,246	4,024	24	26	5,934	7,633	29	31
Core EPS	1.97	2.42	23	24	3.77	4.69	24	27
EPS	1.60	2.07	29	32	2.91	3.91	34	37
Free cash flow	4,615	6,333	37		6,653	9,724	46	

1. Constant currencies (cc), core results and free cash flow are non-IFRS measures. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Continued focus on Free Cash Flow generation

Free Cash Flow[1]

USD billion



2025 growth driven by higher core operating income

1. Free Cash Flow and core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Continuing our shareholder-friendly capital allocation strategy, initiating a new up-to USD 10bn share buyback

Investing in the business

Investments in organic business

Ongoing investment in R&D and CapEx

Value-creating bolt-ons

Acquisition of Regulus Therapeutics

Continued significant capacity for M&A

Substantial cash generation

Returning capital to shareholders

Consistently growing annual dividend[1]

USD 7.8bn dividend paid in H1 2025[2]

Share buybacks

USD 15bn buyback completed in July 2025; initiating up-to USD 10bn buyback to be completed by end 2027

1. In CHF. 2. USD 5.3 billion annual net dividend payment in March, which is the gross dividend of USD 7.8 billion reduced by the USD 2.5 billion Swiss withholding tax that was paid in April 2025, according to its due date.

Raising Novartis 2025 full year core[1] operating income guidance

Expected, barring unforeseen events; growth vs. PY in cc[1]

Net sales

expected to grow
high single-digit

Core operating income

expected to grow
low-teens

(from low double-digit)

Key assumption

- We continue to assume Entresto® US generic entry in mid-2025 for forecasting purposes, though timing of generic entry is subject to ongoing IP and regulatory litigation

FY guidance on other financial KPIs

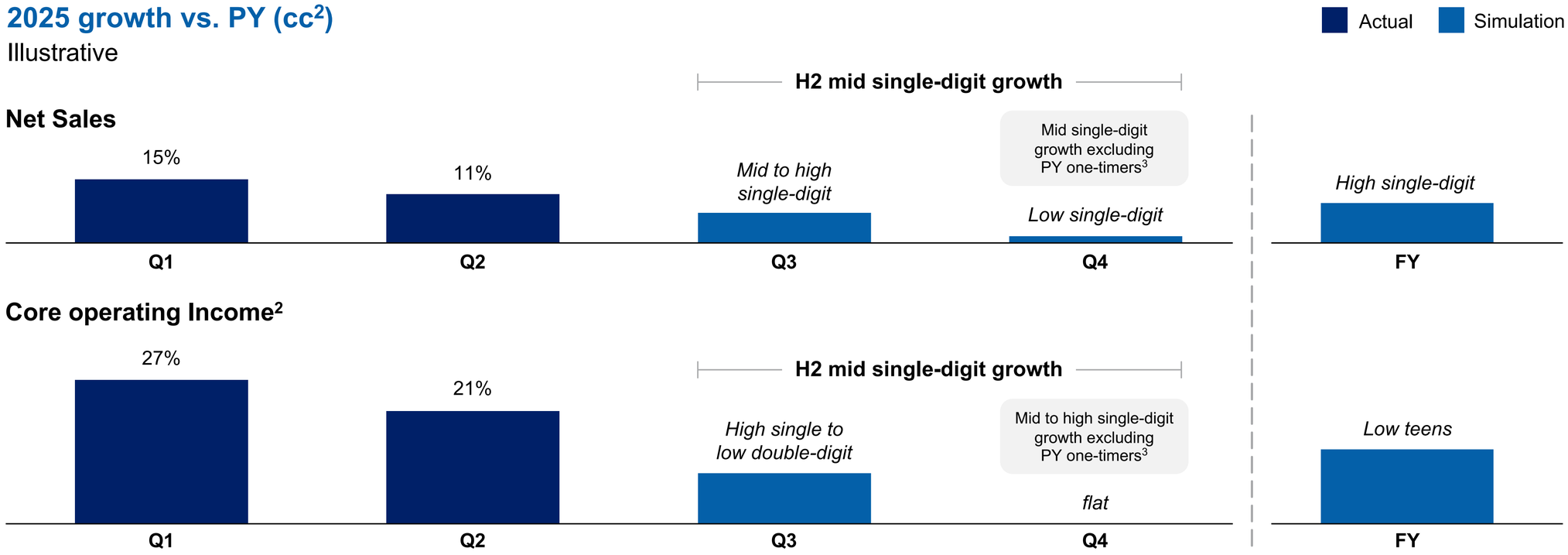
- Core net financial result: Expenses expected to be around USD 1bn
- Core tax rate: Expected to be around 16-16.5%

1. Constant currencies (cc) and core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Strong underlying growth trends expected throughout 2025, with Q4 impacted by prior-year one-timers and potential US generics impact[1]

2025 growth vs. PY (cc[2])

Illustrative

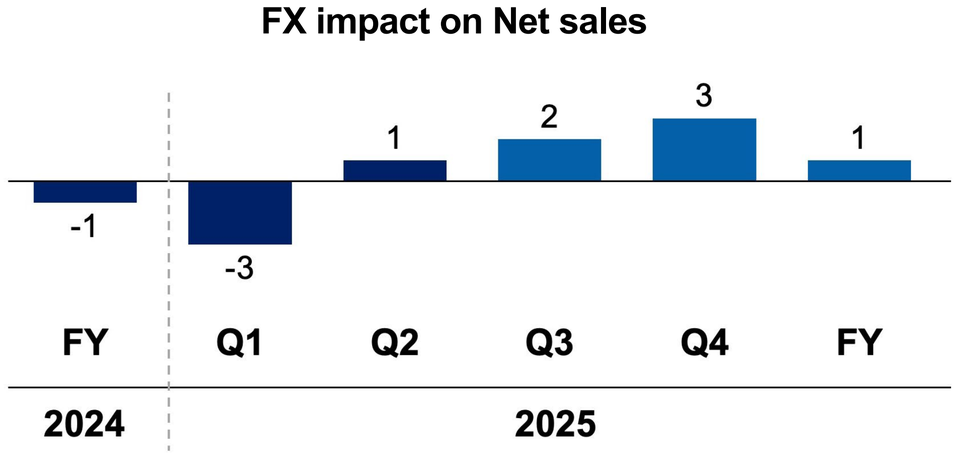


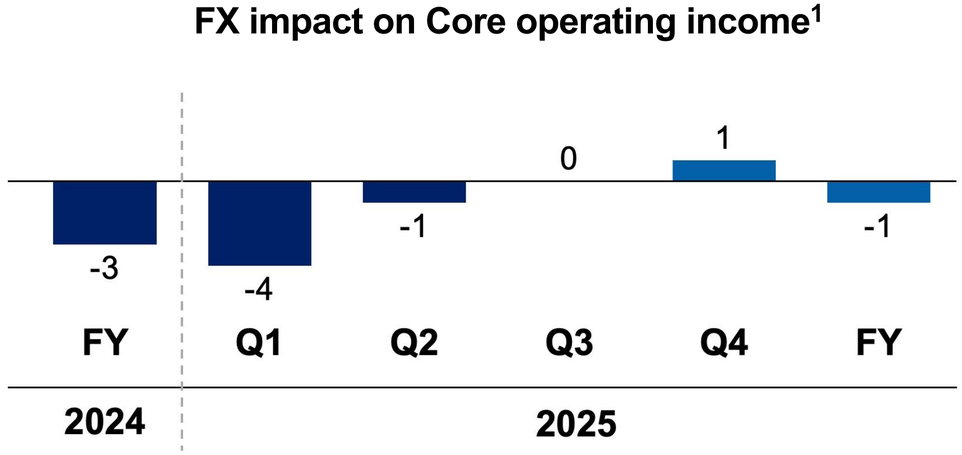
1. Includes potential impact of Entresto US generics, which may change with ongoing Entresto IP and regulatory litigation. on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.
2. Constant currencies (cc) and core results are non-IFRS measures. An explanation of non-IFRS measures can be found
3. Q4 growth impacted by PY gross-to-net true-ups based on invoices for prior periods.

Expected currency impact for full year 2025

Currency impact vs. PY

%pts, assuming mid-July exchange rates prevail in 2025





Actual Simulation

1. Core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Conclusions

Vas Narasimhan, M.D.
Chief Executive Officer

NOVARTIS | Reimagining Medicine

Novartis delivered a strong Q2, with double-digit sales growth and core margin expansion

Key launches are accelerating with consistent strong execution

Continued to advance our pipeline, reaching milestones for both late-stage and emerging assets

Upgraded FY 2025 bottom-line guidance and remain confident in our mid- to long-term growth outlook

Constant currencies (cc) and core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY.

Thank you to Harry for his unwavering commitment and over 22 years at Novartis, and a warm welcome to Mukul to his new role effective March 2026

Harry Kirsch

Retiring and stepping down from the ECN effective March 15, 2026

Mukul Mehta

Appointed Chief Financial Officer and joining the ECN effective March 16, 2026

Appendix

Our pipeline projects at a glance

	Phase I/II	Phase III	Registration	Total
Oncology	24	10	0	34
Solid tumors	20	5	0	25
Hematology	4	5	0	9
Immunology	16	7	1	24
Neuroscience	8	6	1	15
Cardiovascular, Renal and Metabolic	8	8	0	16
Others (thereof IB&GH)	10 (9)	4 (4)	1 (1)	15
	66	35	3	104

IB&GH: In-market Brands and Global Health.

Novartis pipeline in Phase I

17 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)
Solid tumors			
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Breast cancer
			Glioblastoma multiforme
AAA617	Pluvicto®	Radioligand therapy target PSMA	Metastatic neuroendocrine prostate cancer
AAA802	^{225}Ac-PSMA-R2	Radioligand therapy target PSMA	Prostate cancer
ECI830	ECI830	CDK2 inhibitor	Breast cancer
ESP359	ESP359	Radioligand therapy target DLL3	Solid tumors
FXX489	^{177}Lu-NNS309	Radioligand therapy	Solid tumors
HRO761	HRO761	Werner inhibitor	Solid tumors
IAG933	IAG933	-	Mesothelioma
KFA115	KFA115	Novel immunomodulatory Agent	Solid tumors
MGY825	MGY825	-	NSCLC
Hematology			
DFV890	DFV890	NLRP3 inhibitor	Low risk myelodysplastic syndrome
PIT565	PIT565	-	B-cell malignancies

Cardiovascular, Renal and Metabolic

Code	Name	Mechanism	Indication(s)
CYX082	farabursen	MIR17 inhibitor	Autosomal dominant polycystic kidney disease

Neuroscience

Code	Name	Mechanism	Indication(s)
DFT383	DFT383	CTNS gene delivery	Cystinosis
NIO752	NIO752	Tau antisense oligonucleotide	Alzheimer's disease
			Progressive supranuclear palsy
YTB323	rapcabtagene autoleucel	CD19 CAR-T	Relapsing multiple sclerosis
			Primary progressive multiple sclerosis
			Generalized Myasthenia Gravis

Immunology

Code	Name	Mechanism	Indication(s)
IPX643	IPX643	-	Inflammation-driven diseases
PIT565	PIT565	-	Systemic lupus erythematosus
			Rheumatoid arthritis
YTB323	rapcabtagene autoleucel	CD19 CAR-T	Rheumatoid arthritis and severe, refractory Sjögren's disease
YMI024	YMI024	-	Inflammation-driven diseases

Others

Code	Name	Mechanism	Indication(s)
IB&GH			
EDI048	EDI048	CpPI(4)K inhibitor	Cryptosporidiosis
ITU512	ITU512	HbF inducing agent	Sickle cell disease

Novartis pipeline in Phase II

19 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)
Solid tumors			
AAA601	Lutathera®	Radioligand therapy target SSTR	GEPNET, pediatrics
			1L ES-SCLC
			Glioblastoma
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Multiple solid tumors
AAA614	AAA614	Radioligand therapy target FAP	Solid tumors
AAA817	^{225}Ac-PSMA-617	Radioligand therapy target PSMA	Metastatic castration-resistant prostate cancer
DZR123	tulmimetostat	EZH1, EZH2 inhibitor	Solid tumors & lymphomas
JSB462	luxdegalutamide	Androgen receptor protein degrader	Metastatic castration resistant prostate cancer
			Metastatic hormonal sensitive prostate cancer
Hematology			
ABL001	Scemblix®	BCR-ABL inhibitor	Chronic myeloid leukemia, pediatrics
YTB323	rapcabtagene autoleucel	CD19 CAR-T	1L high-risk large B-cell lymphoma

Neuroscience

Code	Name	Mechanism	Indication(s)
HTT227	votoplam	Huntingtin Modulator	Huntington's disease
VHB937	VHB937	TREM2 stabilizer and activator	Amyotrophic lateral sclerosis

Cardiovascular, Renal and Metabolic

Code	Name	Mechanism	Indication(s)
DFV890	DFV890	NLRP3 inhibitor	Cardiovascular risk reduction
LNP023	Fabhalta®	CFB inhibitor	Lupus nephritis
			ANCA associated vasculitis
LTP001	LTP001	SMURF1 inhibitor	Pulmonary arterial hypertension[1]
			Idiopathic pulmonary fibrosis
QCZ484	QCZ484	-	Hypertension
TIN816	TIN816	ATP modulator	Acute kidney injury

1. Phase I / II.

Immunology

Code	Name	Mechanism	Indication(s)
DFV890	DFV890	NLRP3 inhibitor	Osteoarthritis
GHZ339	GHZ339	-	Atopic dermatitis
LOU064	remibrutinib	BTK inhibitor	Food allergy
MAS825	MAS825	IL1B, IL18 Inhibitor	NLRC4-GOF indications
NGI226	NGI226	-	Tendinopathy
RHH646	RHH646	-	Osteoarthritis
VAY736	ianalumab	BAFF-R inhibitor, ADCC-mediated B-cell depletor	Systemic sclerosis
YTB323	rapcabtagene autoleucel	CD19 CAR-T	srSLE/LN
			Systemic sclerosis
			Myositis
			ANCA associated vasculitis

Others

Code	Name	Mechanism	Indication(s)
IB&GH			
EYU688	EYU688	NS4B inhibitor	Dengue fever
INE963	INE963	Plasmodium falciparum inhibitor	Malaria
KAE609	cipargamin	PfATP4 inhibitor	Malaria, severe
			Malaria, uncomplicated
LXE408	LXE408	Proteasome inhibitor	Visceral leishmaniasis
			Chagas
PKC412	Rydapt®	Multi-targeted kinase inhibitor	Acute myeloid leukemia, pediatrics
Others			
LNP023	Fabhalta®	CFB inhibitor	iAMD

Novartis pipeline in Phase III

6 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)
Solid tumors			
AAA601	Lutathera®	Radioligand therapy target SSTR	Gastroenteropancreatic neuroendocrine tumors
AAA617	Pluvicto®	Radioligand therapy target PSMA	Metastatic hormone sensitive prostate cancer (mHSPC)
			Oligometastatic prostate cancer
AAA817	^{225}Ac-PSMA-617	Radioligand therapy target PSMA	Metastatic castration-resistant prostate cancer (mCRPC)
BYL719	Vijoice®	PI3K-alpha inhibitor	Lymphatic malformations
Hematology			
DAK539	pelabresib	BET inhibitor	Myelofibrosis
LNP023	Fabhalta®	CFB inhibitor	Atypical hemolytic uraemic syndrome
VAY736	ianalumab	BAFF-R inhibitor, ADCC-mediated B-cell depletor	1L Immune Thrombocytopenia
			2L Immune Thrombocytopenia
			warm Autoimmune Hemolytic Anemia

Cardiovascular, Renal and Metabolic

Code	Name	Mechanism	Indication(s)
FUB523	zigakibart	Anti-APRIL	IgA nephropathy
KJX839	Leqvio®	siRNA (regulation of LDL-C)	CVRR (secondary prevention)
			CVRR (primary prevention)
			Hyperlipidemia, pediatrics
LNP023	Fabhalta®	CFB inhibitor	C3 glomerulopathy, pediatrics
			IC-MPGN
MAA868	abelacimab	FXI inhibitor	Atrial fibrillation
TQJ230	pelacarsen	ASO targeting Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein (a) (CVRR-Lp(a))

Neuroscience

Code	Name	Mechanism	Indication(s)
BAF312	Mayzent®	S1P1,5 receptor modulator	Multiple sclerosis, pediatrics
LNP023	Fabhalta®	CFB inhibitor	Myasthenia gravis
LOU064	remibrutinib	BTK inhibitor	Multiple sclerosis
			Myasthenia gravis
OMB157	Kesimpta®	CD20 Antagonist	Multiple sclerosis, pediatrics
			Multiple sclerosis, new dosing regimen

Immunology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	Polymyalgia rheumatica
LOU064	remibrutinib	BTK inhibitor	Chronic spontaneous urticaria, pediatrics
			Chronic inducible urticaria
			Hidradenitis suppurativa
VAY736	ianalumab	BAFF-R inhibitor, ADCC-mediated B-cell depletor	Sjögren's disease
			Lupus Nephritis
			Systemic lupus erythematosus

Others

Code	Name	Mechanism	Indication(s)
IB&GH			
AMG334	Aimovig®	CGRPR antagonist	Migraine, pediatrics
KLU156	Ganaplacide + lumefantrine	Non-artemisinin plasmodium falciparum inhibitor	Malaria, uncomplicated
QMF149	Atectura®	LABA + ICS	Asthma, pediatrics
SEG101	Adakveo®	P-selectin inhibitor	Sickle cell disease, pediatrics

Novartis pipeline in registration

1 lead indication

Others

Code	Name	Mechanism	Indication(s)
IB&GH			
RTH258	Beovu®	VEGF Inhibitor	Diabetic retinopathy

Neuroscience

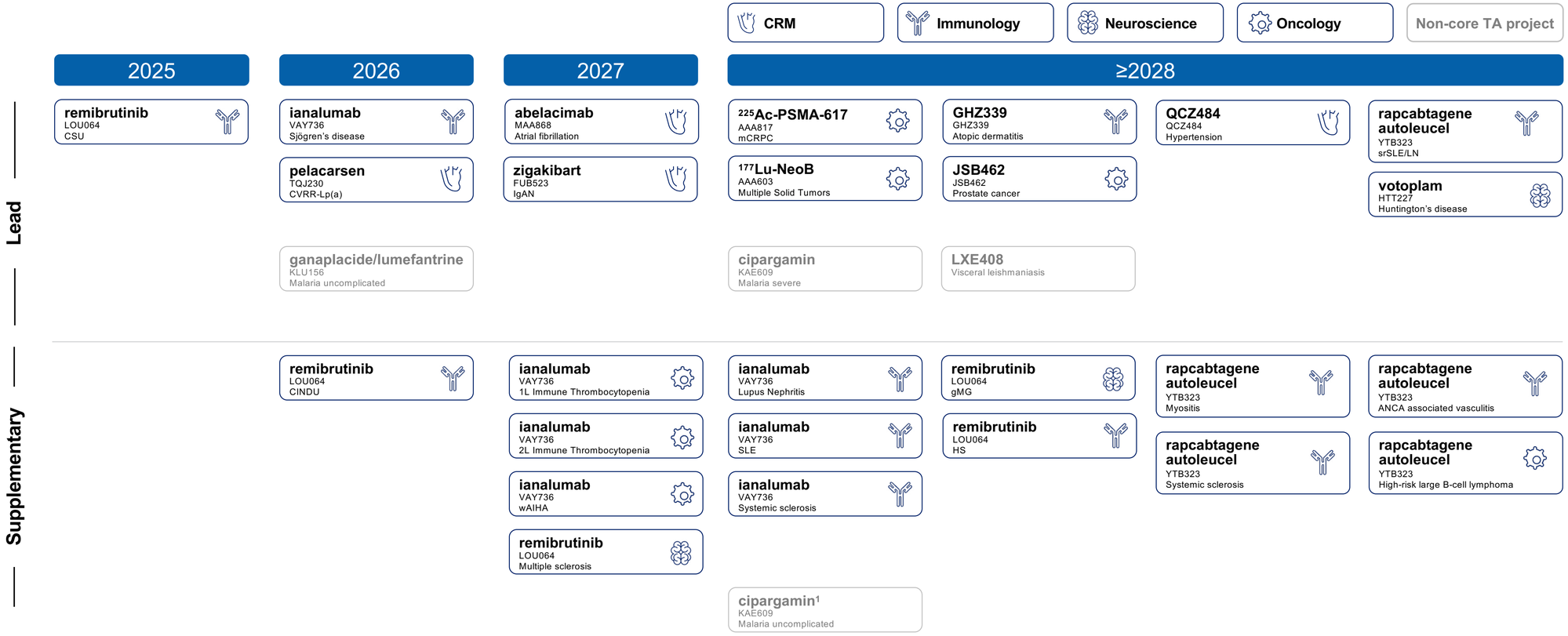
Code	Name	Mechanism	Indication(s)
OAV101	onasemnogene abeparvovec	SMN1 gene replacement therapy	SMA IT administration

Immunology

Code	Name	Mechanism	Indication(s)
LOU064	remibrutinib	BTK inhibitor	Chronic spontaneous urticaria

Novartis submission schedule

New Molecular Entities: Lead and supplementary indications



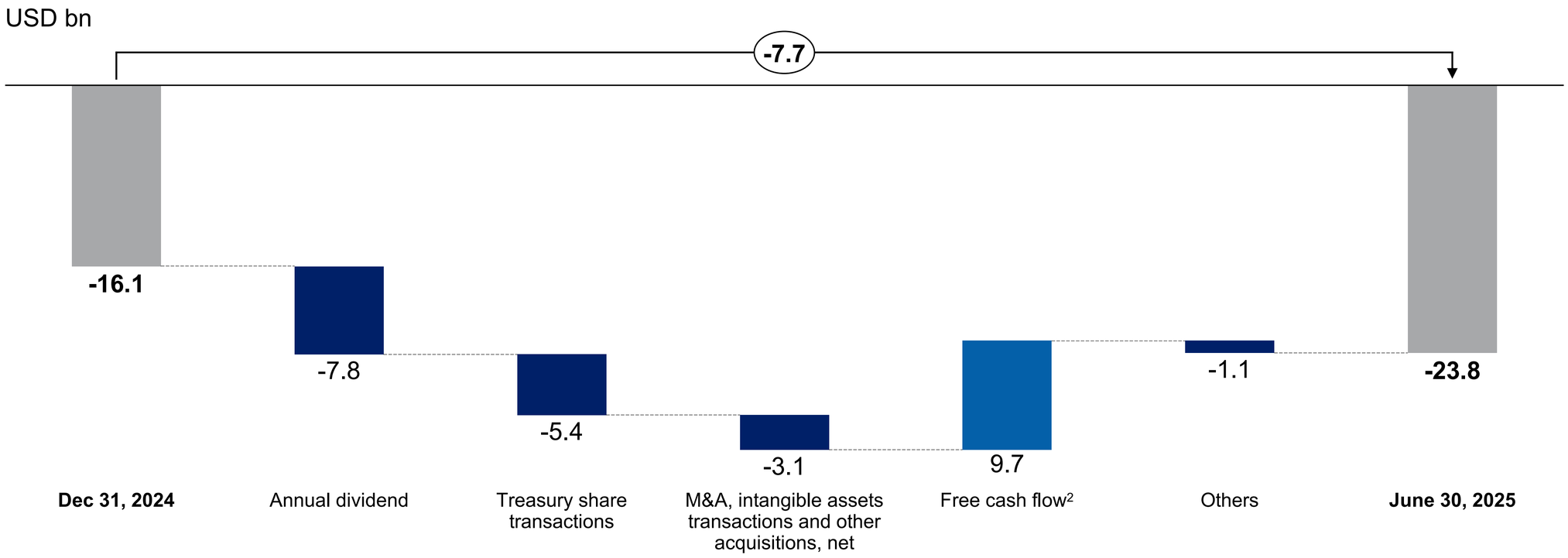
1. Part of triple combination therapy.

Novartis submission schedule

Supplementary indications for existing brands

1. Event-driven trial endpoint. 2. Kesimpta and Mayzent: Pediatric trial in multiple sclerosis run in conjunction (NEOS).

Net debt[1] increased by USD 7.7bn as strong FCF[2] was more than offset by mainly the annual dividend and share buybacks



1. Net debt is presented as additional information. An explanation of additional information can be found on page 47 of the Condensed Interim Financial Report. 2. Free cash flow is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 40 of the Condensed Interim Financial Report.

Clinical Trials Update

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

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

Cardiovascular, Renal and Metabolic

atrasentan - ETA receptor antagonist

NCT04573478 ALIGN (CHK01-01)

Indication	IgA nephropathy
Phase	Phase 3
Patients	380
Primary Outcome Measures	Change in proteinuria Time Frame: Up to Week 24 or approximately 6 months Annualized total estimated Glomerular Filtration Rate (eGFR) slope estimated over 24 months
Arms Intervention	Arm 1 Experimental: Atrasentan, once daily oral administration of 0.75 mg atrasentan for 132 weeks Arm 2 Placebo comparator: Placebo once daily oral administration of placebo for 132 weeks
Target Patients	Patients with IgA nephropathy (IgAN) at risk of progressive loss of renal function
Readout Milestone(s)	2023 (primary endpoint for US initial submission) 2026 (24 months)
Publication	TBD

Fabhalta® - 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	TBD

Fabhalta® - CFB inhibitor

NCT05755386 APPARENT (CLNP023B12302)

Indication	Immune complex-mediated membranoproliferative glomerulonephritis
Phase	Phase 3
Patients	106
Primary Outcome Measures	Log-transformed ratio to baseline in UPCR (sampled from a 24 hour urine collection)
Arms Intervention	Arm 1 experimental: Drug: iptacopan 200 mg b.i.d. (Adults 200mg b.i.d; Adolescents 2x 100mg b.i.d) Arm 2 placebo to iptacopan 200mg b.i.d. (both on top of SoC)
Target Patients	Patients (adults and adolescents aged 12-17 years) with idiopathic IC-MPGN
Readout Milestone(s)	2028
Publication	Vivarelli M, et al., Kidney International Reports (2023), Iptacopan in idiopathic immune complex-mediated membranoproliferative glomerulonephritis: Protocol of the APPARENT multicenter, randomized Phase III study

Leqvio® - siRNA (regulation of LDL-C)

NCT03705234 ORION-4 (CKJX839B12301)

Indication	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH)
Phase	Phase 3
Patients	16124
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 months 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)

NCT05030428 VICTORION-2P (CKJX839B12302)

Indication	Secondary prevention of cardiovascular events in patients with elevated levels of LDL-C
Phase	Phase 3
Patients	16970
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

Leqvio® - siRNA (regulation of LDL-C)

NCT04652726 ORION-16 (CKJX839C12301)

Indication	Hyperlipidemia, pediatrics
Phase	Phase 3
Patients	141
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	Publication Design publication (O-16/-13) in Eur. J. Prev. Cardiol. Vol. 29, Feb. 2022 Presentation at EAS May-2022 on O-13/-16 study design

Leqvio® - siRNA (regulation of LDL-C)

NCT04659863 ORION-13 (CKJX839C12302)

Indication	Hyperlipidemia, pediatrics
Phase	Phase 3
Patients	13
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	Publication Design publication (O-16/-13) in Eur. J. Prev. Cardiol. Vol. 29, Feb. 2022 Presentation at EAS May-2022 on O-13/-16 study design

Leqvio® - siRNA (regulation of LDL-C)

NCT05739383 VICTORION-1P (CKJX839D12302)

Indication	CVRR (Primary prevention)
Phase	Phase 3
Patients	14000
Primary Outcome Measures	Time to the first occurrence of 4P-MACE 4-Point-Major Adverse Cardiovascular Events (4P-MACE): composite of cardiovascular death, non-fatal myocardial infarction, non-fatal ischemic stroke, and urgent coronary revascularization
Arms Intervention	Arm 1 Experimental: Inclisiran Sodium 300mg, subcutaneous injection in pre-filled syringe Arm 2 Placebo
Target Patients	High-risk primary prevention patients
Readout Milestone(s)	2029
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)	2026 (Event driven)
Publication	TBD

QCZ484

NCT06857955 (CQCZ484A12201)

Indication	Hypertension
Phase	Phase 2
Patients	380
Primary Outcome Measures	Change from baseline at Month 3 in mean 24hr systolic blood pressure (SBP) by ambulatory blood pressure measurement (ABPM)
Arms Intervention	Placebo Comparator: Placebo Control Arm 1: QCZ484 Dose 1 solution for injection Arm 2: QCZ484 Dose 2 solution for injection Arm 3: QCZ484 Dose 3 solution for injection Arm 4: QCZ484 Dose 4 solution for injection Arm 5: QCZ484 Dose 5 solution for injection
Target Patients	Mild to moderate hypertensive patients
Readout Milestone(s)	2027
Publication	TBD

zigakibart - Anti-APRIL

NCT05852938 BEYOND (CFUB523A12301)

Indication	IgA nephropathy
Phase	Phase 3
Patients	350
Primary Outcome Measures	Change in proteinuria [Time Frame: 40 weeks or approximately 9 months]
Arms Intervention	Arm 1 Experimental: BION-1301 (Zigakibart) 600mg subcutaneous administration every 2 weeks for 104 weeks Arm 2 Placebo Comparator: Placebo subcutaneous administration every 2 weeks for 104 weeks
Target Patients	Adults with IgA Nephropathy
Readout Milestone(s)	2026
Publication	WCN Poster April 2024: BEYOND: A Phase 3, Randomized, Double-Blind, Placebo-controlled Trial of Zigakibart in Adults with IgA Nephropathy. Trimarchi H., et. al.

Immunology

Cosentyx® - IL-17A inhibitor

NCT05767034 REPLENISH (CAIN457C22301)

Indication	Polymyalgia rheumatica
Phase	Phase 3
Patients	360
Primary Outcome Measures	Proportion of participants achieving sustained remission
Arms Intervention	Arm 1 Experimental: Secukinumab 300 mg, randomized in 1:1:1 ratio every 4 weeks Arm 2 Experimental: Secukinumab 150 mg, randomized in 1:1:1 ratio every 4 weeks Arm 3 Placebo : randomized in 1:1:1 ratio every 4 weeks
Target Patients	Adult patients with PMR who have recently relapsed
Readout Milestone(s)	2025
Publication	TBD

Cosentyx® - IL-17A inhibitor

NCT04930094 GCAPTAIN (CAIN457R12301)

Indication	Giant cell arteritis
Phase	Phase 3
Patients	349
Primary Outcome Measures	Number of participants with sustained remission
Arms Intervention	Experimental: Secukinumab 150 and 300 mg Placebo Comparator: Placebo
Target Patients	Patients with Giant Cell Arteritis (GCA)
Readout Milestone(s)	Primary 2025(actual) Study did not meet primary endpoint
Publication	TBD

GHZ339

NCT06947993 (CADPT17A12201)

Indication	Atopic dermatitis
Phase	Phase 2
Patients	224
Primary Outcome Measures	Change from baseline in the Eczema Area and Severity Index (EASI) score at Week 16. EASI will be used to assess the extend and severity of atopic dermatitis on a scale from 0 to 72 where 72 is worst eczema.
Arms Intervention	-Experimental: GHZ339 Dose A, Participants who will receive GHZ339 at dose A during Treatment Period 1 will receive GHZ339 at dose A during Treatment Period 2 -Experimental: GHZ339 Dose B, Participants who will receive GHZ339 at dose B during Treatment Period 1 will receive GHZ339 at dose B during Treatment Period 2 -Experimental: GHZ339 Dose C. Participants who will receive GHZ339 at dose C during Treatment Period 1 will receive GHZ339 at dose C or A during Treatment Period 2 -Experimental: GHZ339 Dose D. Participants who will receive GHZ339 at dose D during Treatment Period 1 will receive GHZ339 at dose D or A during Treatment Period 2 -Placebo Comparator: Placebo. Participants who will receive placebo during Treatment Period 1 will receive GHZ339 at dose A during Treatment Period 2 2
Target Patients	Patients with moderate to severe Atopic Dermatitis
Readout Milestone(s)	Primary 2029
Publication	TBD

ianalumab - BAFF-R inhibitor, ADCC-mediated B-cell depletor

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: Experiemental - 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, ADCC-mediated B-cell depletor

NCT05349214 NEPTUNUS-2 (CVAY736A2302)

Indication	Sjögren's disease
Phase	Phase 3
Patients	506
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 disease
Readout Milestone(s)	Primary 2025
Publication	TBD

ianalumab - BAFF-R inhibitor, ADCC-mediated B-cell depletor

NCT05350072 NEPTUNUS-1 (CVAY736A2301)

Indication	Sjögren's disease
Phase	Phase 3
Patients	276
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 disease
Readout Milestone(s)	Primary 2025
Publication	TBD

ianalumab - BAFF-R inhibitor, ADCC-mediated B-cell depletor

NCT05639114 SIRIUS-SLE 1 (CVAY736F12301)

Indication	Systemic lupus erythematosus
Phase	Phase 3
Patients	406
Primary Outcome Measures	Proportion of participants on monthly ianalumab achieving Systemic Lupus Erythematosus Responder Index -4 (SRI-4) [Time Frame: Week 60]
Arms Intervention	Experimental: Ianalumab s.c. monthly Experimental: Ianalumab s.c. quarterly Placebo Comparator: Placebo s.c. monthly
Target Patients	Patients with active systemic lupus erythematosus (SLE)
Readout Milestone(s)	2027
Publication	TBD

ianalumab - BAFF-R inhibitor, ADCC-mediated B-cell depletor

NCT05624749 SIRIUS-SLE 2 (CVAY736F12302)

Indication	Systemic lupus erythematosus
Phase	Phase 3
Patients	280
Primary Outcome Measures	Proportion of participants achieving Systemic Lupus Erythematosus Responder Index -4 (SRI-4) [Time Frame: Week 60]
Arms Intervention	Experimental: ianalumab s.c. monthly Placebo Comparator: placebo s.c. monthly
Target Patients	Patients with active systemic lupus erythematosus (SLE)
Readout Milestone(s)	2027
Publication	TBD

ianalumab - BAFF-R inhibitor, ADCC-mediated B-cell depletor

NCT06470048 (CVAY736S12201)

Indication	Systemic sclerosis
Phase	Phase 2
Patients	200
Primary Outcome Measures	3/5 Revised Composite Response Index in Systemic Sclerosis 25 (rCRISS25) response at Week 52
Arms Intervention	Arm 1 Experimental VAY736 (Ianalumab) - Treatment Period 1: Ianalumab subcutaneous (s.c.) injection as defined in the protocol - Treatment Period 2: Open-label (OL) Ianalumab subcutaneous (s.c.) injection as defined in the protocol Arm 2 Placebo Comparator: Placebo - Treatment Period 1: Placebo to Ianalumab subcutaneous (s.c.) injection as defined in the protocol - Treatment Period 2: Open-label (OL) Ianalumab subcutaneous (s.c.) injection as defined in the protocol
Target Patients	Patients with diffuse cutaneous systemic sclerosis
Readout Milestone(s)	2028
Publication	TBD

remibrutinib - BTK inhibitor

NCT05976243 (CLOU064M12301)

Indication	Chronic inducible urticaria
Phase	Phase 3
Patients	348
Primary Outcome Measures	1. Proportion of participants with complete response in Total Fric Score; symptomatic dermographism [Time Frame: Week 12] 2. Proportion of participants with complete response in critical temperature threshold; cold urticaria [Time Frame: Week 12] 3. Proportion of participants with itch numerical rating scale =0; cholinergic urticaria [Time Frame: Week 12]
Arms Intervention	All arms oral, twice daily: Arm 1 Experimental Remibrutinib, symptomatic dermographism group Arm 2 Placebo symptomatic dermographism group Arm 3 Experimental Remibrutinib, cold urticaria group Arm 4 Placebo cold urticaria group Arm 5 Experimental Remibrutinib, cholinergic urticaria group Arm 6 Placebo cholinergic urticaria group
Target Patients	Adults suffering from CINDU inadequately controlled by H1-antihistamines
Readout Milestone(s)	2026
Publication	TBD

remibrutinib - BTK inhibitor

NCT06799000 RECHARGE1 (CLOU064J12301)

Indication	Hidradenitis suppurativa
Phase	Phase 3
Patients	555
Primary Outcome Measures	Proportion of participants with Hidradenitis Suppurativa clinical response 50 (HiSCR50) at Week 16
Arms Intervention	Arm 1: Experimental Participants randomized to receive remibrutinib Dose A during Treatment Period 1 and 2 Arm 2: Experimental Participants randomized to receive remibrutinib Dose B during Treatment Period 1 and 2 Arm 3: Placebo comparator Participants randomized to receive placebo during Treatment Period 1 followed by remibrutinib dose B during Treatment Period 2
Target Patients	Adult patients With moderate to severe Hidradenitis Suppurativa
Readout Milestone(s)	2028
Publication	TBD

remibrutinib - BTK inhibitor

NCT06840392 RECHARGE2 (CLOU064J12302)

Indication	Hidradenitis suppurativa
Phase	Phase 3
Patients	555
Primary Outcome Measures	Proportion of participants with Hidradenitis Suppurativa clinical response 50 (HiSCR50) at Week 16
Arms Intervention	Arm 1: Experimental Participants randomized to receive remibrutinib Dose A during Treatment Period 1 and 2 Arm 2: Experimental Participants randomized to receive remibrutinib Dose B during Treatment Period 1 and 2 Arm 3: Participants randomized to receive placebo during Treatment Period 1 followed by remibrutinib dose B during Treatment Period 2
Target Patients	Adult patients With moderate to severe Hidradenitis Suppurativa
Readout Milestone(s)	2028
Publication	TBD

Neuroscience

Fabhalta® - CFB inhibitor

NCT123456 APPRAISE (CLNP023Q12301)

Indication	Generalized Myasthenia Gravis
Phase	Phase 3
Patients	146
Primary Outcome Measures	Change from baseline to Month 6 in Myasthenia Gravis Activity of Daily Living (MG-ADL) total score
Arms Intervention	Participants who meet the eligibility criteria will be randomized in a ratio of 1:1, to receive either iptacopan at a dose of 200 mg orally b.i.d or matching placebo
Target Patients	Patients with generalized MG who anti-AchR-positive and are not adequately responding to 2/3rd line SoC.
Readout Milestone(s)	2027
Publication	TBD

Kesimpta® - anti-CD20

NCT06869785 FILIOS (COMB157Q12301)

Indication	Multiple sclerosis new dosing regimen
Phase	Phase 3
Patients	180
Primary Outcome Measures	Ofatumumab plasma pharmacokinetics - area under the curve, up to 12 weeks
Arms Intervention	Arm 1: Active Comparator Ofatumumab dose 1, Approved dosage Arm 2: Experimental Ofatumumab dose 2, New dosage
Target Patients	Patients with relapsing multiple sclerosis
Readout Milestone(s)	2028
Publication	TBD

Mayzent® - S1P1,5 receptor modulator

NCT04926818 NEOS (CBAF312D2301)

Indication	Multiple sclerosis, pediatrics
Phase	Phase 3
Patients	120
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 120 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)	2027
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

remibrutinib - BTK inhibitor

NCT06744920 RELIEVE (CLOU064O12301)

Indication	Myasthenia Gravis
Phase	Phase 3
Patients	180
Primary Outcome Measures	Change from baseline to Month 6 in Myasthenia Gravis Activity of Daily Living (MG-ADL) total score
Arms Intervention	Arm 1 experimental: remibrutinib tablet taken orally Arm 2 placebo comparator: placebo tablet taken orally
Target Patients	Patients with generalized Myasthenia Gravis
Readout Milestone(s)	2028
Publication	TBD

Oncology

225Ac-PSMA-617 - Radioligand therapy target PSMA

NCT06780670 AcTFirst (CAAA817B12301)

Indication	Metastatic castration-resistant prostate cancer
Phase	Phase 3
Patients	605
Primary Outcome Measures	Radiographic Progression Free Survival (rPFS)
Arms Intervention	Arm 1: Investigational Arm, AAA817+ARPI (enzalutamide or abiraterone) Participants will receive AAA817 infusion directly into a vein with ARPIs. Arm 2: Investigational Arm, AAA817 Participants will receive AAA817 infusion directly into a vein. Arm 3: Control arm, Investigator's choice of SoC (ARPI or taxane-based chemotherapy) Participants will receive standard treatment as decided by the trial doctor either as a chemotherapy infusion directly into a vein or ARPI either as capsules or tablets.
Target Patients	Adult participants with PSMA-positive metastatic Castration Resistant Prostate Cancer (mCRPC)
Readout Milestone(s)	2028
Publication	TBD

ianalumab - BAFF-R inhibitor, ADCC-mediated B-cell depletor

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)	2026
Publication	TBD

ianalumab - BAFF-R inhibitor, ADCC-mediated B-cell depletor

NCT05653219 VAYHIT2 (CVAY736Q12301)

Indication	2L Immune Thrombocytopenia
Phase	Phase 3
Patients	152
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, ADCC-mediated B-cell depletor

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 Comparator (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	75
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)	2028
Publication	TBD

Lutathera® - Radioligand therapy target SSTR

NCT06784752 NETTER-3 (CAAA601A62301)

Indication	Gastroenteropancreatic neuroendocrine tumors
Phase	Phase 3
Patients	240
Primary Outcome Measures	Progression Free Survival (PFS) centrally assessed by Blinded Independent Review Committee (BIRC)
Arms Intervention	Arm 1: Experimental: [177Lu]Lu-DOTA-TATE + Octreotide LAR Participants in this arm will receive [177Lu]Lu-DOTA-TATE plus Octreotide long-acting release (LAR). Arm 2: Active Comparator: Octreotide LAR Participants in this arm will receive Octreotide LAR only.
Target Patients	Patients newly diagnosed with Grade 1 and Grade 2 (Ki-67 <10%) advanced GEP-NET with high disease burden
Readout Milestone(s)	2028
Publication	TBD

luxdegalutamide - Androgen receptor protein degrader

NCT07047118 (CJSB462B12201)

Indication	Metastatic castration resistant prostate cancer
Phase	Phase 2
Patients	130
Primary Outcome Measures	Efficacy: Achieving Prostate Specific Antigen 50 (PSA50) Rate. Time Frame: From Baseline at any point (confirmed by a 2nd PSA ≥ 3wks without progression in between) Safety: Incidence rate of adverse events (AEs). Tolerability: Number of participants with dose adjustments & Duration of exposure to study treatment. Time Frame: From Baseline till 30 days safety follow-up post-EOT.
Arms Intervention	Experimental: Arm 1, JSB462 100 mg QD + AAA617 7.4 GBq Q6W Experimental: Arm 2, JSB462 300 mg QD + AAA617 7.4 GBq Q6W Active Comparator: Arm 3, AAA617 7.4 GBq Q6W
Target Patients	Adult male patients with PSMA-positive Metastatic Castration Resistant Prostate cancer (mCRPC)
Readout Milestone(s)	2030
Publication	TBD

NCT06991556 (CJSB462C12201)

Indication	Metastatic hormonal sensitive prostate cancer
Phase	Phase 2
Patients	150
Primary Outcome Measures	Efficacy: Prostate Specific Antigen 90 (PSA90) Rate. Time Frame: From Baseline at any point (confirmed by a 2nd PSA ≥ 3wks without progression in between) Safety: Incidence rate of adverse events (AEs). Tolerability: Number of participants with dose adjustments & Duration of exposure to study treatment. Time Frame: From Baseline till 30 days safety follow-up post-EOT.
Arms Intervention	Experimental: Arm 1, JSB462 100 mg QD + abiraterone 1000 mg QD Experimental: Arm 2, JSB462 300 mg QD + abiraterone 1000 mg QD Active Comparator: Arm 3, abiraterone 1000 mg QD or enzalutamide 160 mg QD
Target Patients	Adult male patients with Metastatic Hormone-Sensitive Prostate Cancer (mHSPC)
Readout Milestone(s)	2032
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: 2025 (actual). PSMAddition met its primary endpoint with a statistically significant and clinically meaningful benefit in rPFS in patients treated with Pluvicto plus SoC versus SoC alone
Publication	TBD

Vijoice® - PI3Ki

NCT05948943 EPIK-L1 (CBYL719P12201)

Indication	Lymphatic Malformation
Phase	Phase 2/3
Patients	230
Primary Outcome Measures	Stage 2: Radiological response rate at Week 24 of Stage 2 (adult and pediatric (6 - 17 years of age) participants) Time Frame: Baseline, Week 24
Arms Intervention	Arm 1: Experimental. Adult participants, alpelisib dose 1 (Stage 1) Arm 2: Experimental. Adult participants, alpelisib dose 2 (Stage 1) Arm 3: Experimental. Pediatric participants (6-17 years of age), alpelisib dose 2 (Stage 1) Arm 4: Experimental. Pediatric participants (6-17 years of age), alpelisib dose 3 (Stage 1) Arm 5: Experimental. Adult participants, alpelisib (Stage 2) Arm 6: Placebo comparator. Adult participants, placebo (Stage 2) Arm 7: Experimental. Pediatric participants (6-17 years of age), alpelisib (Stage 2) Arm 8: Placebo Comparator. Pediatric participants (6-17 years of age), placebo (Stage 2) Arm 9: Experimental. Pediatric participants (2-5 years of age), alpelisib (Stage 2)
Target Patients	Pediatric and adult patients with lymphatic malformations associated with a PIK3CA mutation
Readout Milestone(s)	2030
Publication	TBD

In-market Brands & Global Health

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	Age descending treatment evaluating IV KAE609 doses versus active comparator, IV Artesunate. Follow on therapy for all arms: Coartem, Standard of care
Target Patients	Patients with Malaria, severe
Readout Milestone(s)	2025
Publication	TBD

ganaplacide/lumefantrine - Non-artemisinin plasmodium falciparum inhibitor

NCT05842954 KALUMA (CKLU156A12301)

Indication	Malaria, uncomplicated
Phase	Phase 3
Patients	1720
Primary Outcome Measures	PCR-corrected adequate clinical and parasitological response (ACPR) at day 29
Arms Intervention	Arm 1 experimental: KLU156 oral; 400/480 mg (ganaplacide/ lumefantrine) is the fixed dose combination for patients with a bodyweight ≥ 35kg. Patients < 35kg will take a fraction of the dose according to weight group as defined in the protocol. Arm 2 active comparator: Coartem, oral, dosing will be selected based on patient's body weight as per product's label.
Target Patients	Adults and children ≥ 10 kg Body Weight with uncomplicated P. Falciparum Malaria including mixed infection
Readout Milestone(s)	2025
Publication	TBD

Rydapt® - Multi-targeted kinase inhibitor

NCT03591510 (CPKC412A2218)

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

Abbreviations

Abbreviation	Full Form
AAV	Anti-neutrophil cytoplasmic antibody (ANCA)-associated vasculitis
ACC	American College of Cardiology
ACS	Acute Coronary Syndrome
AD	Alzheimer's Disease
ADT	Androgen Deprivation Therapy
AES	European Atherosclerosis Society
AHA	American Heart Association
ARPI	Androgen Receptor Pathway Inhibitors
AS	Ankylosing Spondylitis
ASCO	American Society of Clinical Oncology
C3G	Complement 3 Glomerulopathy
CIndU	Chronic Inducible Urticaria
CML	Chronic Myeloid Leukemia
CSU	Chronic Spontaneous Urticaria
CVA	Cerebrovascular Accident
DMT	Disease-Modifying Therapies
eBC	Early Breast Cancer
EHA	European Hematology Association
ESMO	European Society For Medical Oncology
EULAR	European Alliance of Associations for Rheumatology
FA	Food Allergy
GCA	Giant Cell Arteritis
GEP-NET	Gastroenteropancreatic Neuroendocrine Tumors
gMG	Generalized Myasthenia Gravis
Hb	Hemoglobin
HCP	Health Care Provider
HD	Huntington's Disease
HS	Hidradenitis Suppurativa
HTN	Hypertension
IB&GH	In-market Brands and Global Health
IgAN	Immunoglobin A Nephropathy
IIM	Idiopathic Inflammatory Myopathies
ITP	Immune Thrombocytopenia
IV	Intravenous
LN	Lupus Nephritis

Abbreviation	Full Form
LoE	Loss of Exclusivity
mBC	Metastatic Breast Cancer
mCRPC	Metastatic Castration-Resistant Prostate Cancer
mHSPC	Metastatic Hormone-Sensitive Prostate Cancer
MOTRx	Units Normalized to Month-on-Therapy
MS	Multiple Sclerosis
NBRx	New to Brand Prescription
NCCN	National Comprehensive Cancer Network
NLA	National Lipid Association
nr-axSpA	Non-Radiographic Axial Spondyloarthritis
NSCLC	Non-Small Cell Lung Cancer
OS	Overall Survival
PC	Prostate Cancer
PFS	Progression-Free Survival
PMR	Polymyalgia Rheumatica
pMS	Progressive Multiple Sclerosis
PsA	Psoriatic Arthritis
PSMA	Prostate-Specific Membrane Antigen
PsO	Psoriasis
RA	Rheumatoid Arthritis
RDP	Regulatory Data Protection
REMS	Risk Evaluation and Mitigation Strategy
RMS	Relapsing Multiple Sclerosis
rPFS	Radiographic Progression-Free Survival
Scr	Screening
SE	Standard Error
SjD	Sjogren's Disease
SLE	Systemic Lupus Erythematosus
SLEDAI-2K	Systemic Lupus Erythematosus Disease Activity Index 2000
SMA	Spinal Muscular Atrophy
SpA	Spondyloarthritis
srSLE	Severe Refractory Systemic Lupus Erythematosus
srSLE/LN	Severe Refractory Systemic Lupus Erythematosus/Lupus Nephritis
SSc	Systemic Sclerosis
TRx	Total Prescriptions

References 1 of 3

Kisqali® (slide 6 references)

1	IQVIA Market Sizing Monthly Report, May 2025; Data lag: ~ 2 months.
2	Of CDK4/6 market, US rolling 3 months ending May 2025, IQVIA Breast Cancer Market Sizing report.
3	BEST, NBRx (EU5, AU, KR, CA) as of Apr 2025, TRx top 9 countries (EU5, AU, KR, CA, BR) as of Mar 2025.
4	eBC DE NBRx share from BEST as of Apr 2025.

Kesimpta® (slide 7 references)

1	TRx adjusted data: Contracted SP data + access card and IQVIA NPA adjusted by NSP. Based on availability; Jun actuals through Jun 6, 2025, and projected for remaining 3 weeks of Jun 2025.
2	IQVIA NSP/NPA and Kesimpta NVS SP and copay claims (May 2024 – Apr 2025).
3	The 8 markets include Germany, Japan, China, Australia, Canada, France, Italy, and UK.
4	IQVIA MIDAS volume data, converted to patient equivalents using standard dosing assumptions.
5	As per stability technical specification data, when the patient is ready to inject, it typically takes less than 1 minute a month to administer. Once-monthly dosing begins after the initial dosing period, which consists of 20 mg subcutaneous doses at weeks 0, 1, and 2. Please see Instructions for Use for more detailed instructions on preparation and administration of KESIMPTA. Patient must take pen out of the refrigerator 15-30 minutes before self-administering.

Pluvicto® (slide 8 references)

1	Data as of May 2025, based on internal ordering system and analysis.
2	NBRx = new patient doses and TRx = total patient doses.

PSMAddition (slide 9 references)

1	Treatment until radiographic progression or no longer clinically benefiting. Crossover to 177Lu-PSMA-617 + SoC allowed upon radiographic progression confirmed by BIRC.
2	Up to 45 days of ADT and/or an ARPI for metastatic prostate cancer prior to screening. Positive lesions defined as having [68Ga]Ga-PSMA-11 uptake higher than non-cancerous liver parenchyma by visual assessment. Lesion(s) can be in any system. PSMA expression qualification will be determined by central readers.

References 2 of 3

Leqvio® (slide 10 references)

1 Includes PCSK9 monoclonal antibodies and bempedoic acid.
2 MOTRx Q2 QTD ending 20-Jun-2025 vs. PY.
3 800+ Priority Health Systems, depth growth Q2 2025 vs. PY.
4 Rao, S, O'Donoghue, M, Ruel, M. et al. 2025 ACC/AHA/ACEP/NAEMSP/SCAI Guideline for the Management of Patients With Acute Coronary Syndromes: A Report of the American College of Cardiology/American Heart Association Joint Committee on Clinical Practice Guidelines. JACC. 2025 Jun, 85 (22) 2135–2237. https://doi.org/10.1016/j.jacc.2024.11.009.

Scemblix® (slide 11 references)

1 US March rolling 3-months US IQVIA CML market sizing report, May 2025.
2 For Q2 2025 International Patient share calculation, considered individual markets patient shares as follows – EU4: IQVIA OD until Feb 2025 (preliminary data), Germany: LRx until Apr 2025 and Japan: MDV until Q1 2025, and assumed same shares for Q2 2025 as in Q1 2025.

Cosentyx® (slide 12 references)

1 IQVIA National Source of Business (NSOB) data. NBRx volume has been adjusted by excluding the volume of Cordavis Humira since Mar 8, 2024.
2 IV formulation indication: PsA, AS, nr-axSpA. Source: IQVIA mastered 867 data.
3 Refers to EU5. Indications: Pso, HS, PsA, axSpA. For EU: France - IQVIA (Mar 2025); UK - IQVIA, Stethos (Apr 2025); Germany - IQVIA (Apr 2025); Italy - Stethos (Apr 2025), Elma; Spain - Amber market research data, IQVIA (Dec 2024).
4 Hospital value (sales, growth and share). Market definition includes "all approved immunology brands with at least one indication overlapping with Cosentyx". Source: IQVIA CHPA (Apr 2025).

Entresto® (slide 13 references)

1 Novartis will continue to appropriately enforce its US IP and regulatory rights around Entresto®.
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 CHF and HTN (JP). HTN is not an approved indication in the US and EU.
3 Based on 2024 sales.
4 Extension of regulatory data protection to November 2026 in EU based on approval of pediatric indication.

References 3 of 3

Renal portfolio (slide 14 references)

1 Fabhalta prescribed patients. There is no data on the impact of Fabhalta on glomerular inflammation.
2 Approved by FDA in Apr 2025. All information specific to US launch.
3 Use of Vanrafia is contraindicated in patients who are pregnant and patients with hypersensitivity. Serious warnings associated with Vanrafia include embryo-fetal toxicity, heptatoxicity, fluid retention, and decreased sperm counts. (Heerspink HJL, Jardine M, Kohan DE, et al. Atrasentan in Patients with IgA Nephropathy. N Engl J Med. 2025;392(6):544-554. doi:10.1056/NEJMoa2409415).
4 Commercial lives; SPP Shipment & Dispense Data launch to date through Jun 20, NPS + D2SP.

Remibrutinib (slide 15 references)

1 Martinis MD, et al. Arch Immunol Ther Exp. 2020; 68:8.
2 Warren CM, et al. Curr Allergy Asthma Rep. 2020; 6:1−9.

YTB323 (slide 16 references)

1 Rapcabtagene autoleucel.
2 Data presented at EULAR 2025.
3 Mean line with standard error bars.
4 SLEDAI-2K items with no activity at screening in any patients are not shown.
5 The mean disease duration in the EULAR 2025 data was 13.6 years. Extended disease duration particularly in LN patients leads to a greater likelihood for irreversible kidney damage.

YTB323 (slide 17 references)

1 Basket study design. Patients have a single disease rather than comorbid conditions.
2 Intended to be registration-enabling.