Q1 2022 Results
Investor presentation

NOVARTIS | Reimagining Medicine

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Vas Narasimhan

Chief Executive Officer

Company overview

Novartis off to a solid start in Q1 across our value drivers

Growth 1

Group sales **+5%** cc

IM sales **+4%** cc

Sandoz sales **+8%** cc

Innovation 3

Pluvicto® mCRPC post-taxane approved in US

Vijoice® PROS approved in US[1]

Beovu® DME approved in EU

Kymriah® r/r follicular lymphoma EU/EEA CHMP positive opinion

JDQ443 encouraging early clinical data from Ph1b KontRASt-01 study[1]

Productivity 2

Group core operating income **+9%** cc

IM core operating income **+5%** cc

IM core margin 35.9% (**+0.2%**pts) cc

Sandoz core operating income **+26**% cc

ESG 4

AMR: extension / expansion of collaboration agreement with Ares Genetics, enabling genomic surveillance for resistant pathogens[1]

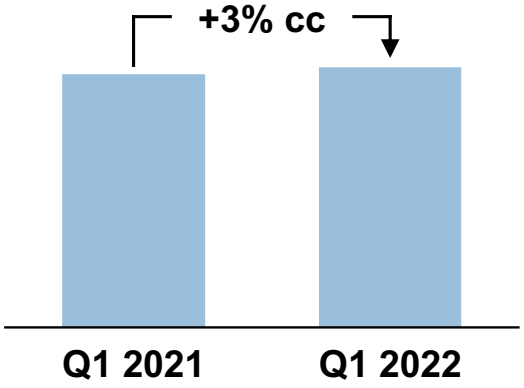
Access: agreements signed in Zambia, Tanzania (e.g. SCD, HF, HTN)

Constant currencies (cc), core results are non-IFRS measures; explanation can be found on page 35 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY. IM – Innovative Medicines division. 1. Post quarter event.

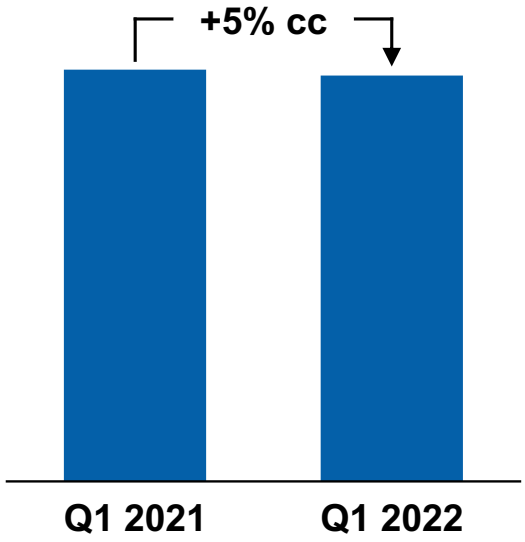
Q1 Innovative Medicines (IM) sales grew across US and ex-US, driven by our in-market growth drivers

IM sales USD 10.2bn (+4% cc)

US | Q1 2022 USD 3.7bn

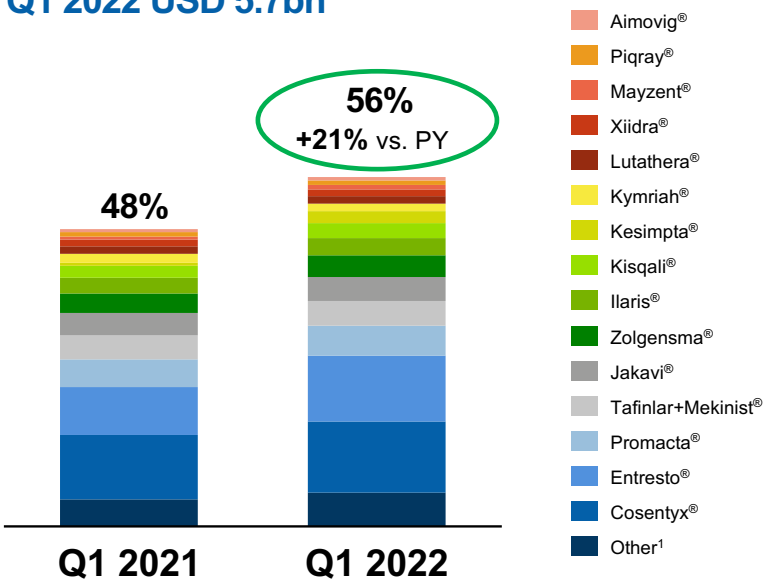


Ex-US | Q1 2022 USD 6.5bn



Growth drivers +21% cc, 56% of IM sales

Q1 2022 USD 5.7bn



All % growth relate to cc unless otherwise stated 1. Includes Xolair®, Beovu®, Adakveo®, Tabrecta®, Scemblix®, Enerzair®, Atectura® , Leqvio ®, Luxturna and Pluvicto®.

Strong performance of Entresto®, Kesimpta®, Cosentyx®, Zolgensma®, Kisqali® and launching Leqvio®...

Q1 sales[1]

	Sales USD million	Growth vs. PY USD million	Growth vs. PY cc
Entresto	1,093	304	42%
Kesimpta	195	145	nm
Cosentyx	1,159	106	12%
zolgensma	363	44	18%
KISQALI	239	44	28%
Xolair	368	33	17%
ILARIS	285	29	18%
PROMACTA	491	28	9%
JAKAVI	389	26	14%
SCEMBLIX	25	25	nm
MAYZENT	79	24	47%
LEQVIO	14	13	nm

Constant currencies (cc) is a non-IFRS measure; explanation of non-IFRS measures can be found on page 35 of Condensed Interim Financial Report. Unless otherwise noted, all growth rates refer to same period in PY
nm – not meaningful 1. Innovative Medicines division

… reinforcing our confidence in mid-term growth outlook

Q1 sales

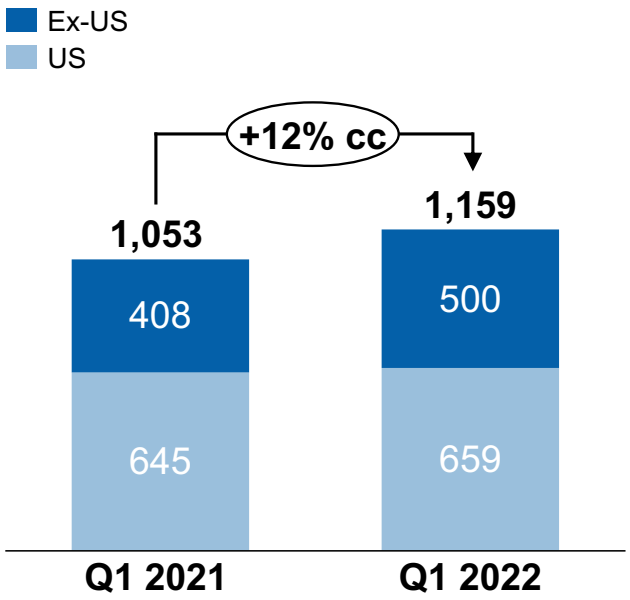
Cosentyx®	Entresto®	zolgensma®	KISQALI®	Kesimpta®	LEQVIO®
USD 1.2 bn	USD 1.1 bn	USD 0.4 bn	USD 0.2 bn	USD 0.2 bn	nm
+12%	+42%	+18%	+28%	nm	nm
Est. CAGR (2020-26) Low double digit	Est. CAGR (2020-26) Double digit until LoE	Est. CAGR (2020-26) Low to mid teens	Est. CAGR (2020-26) Low 30s[2]	Est. CAGR (2020-26) nm	Est. CAGR (2020-26) nm
Peak sales **USD >7bn**	Peak sales **USD >5bn**	Peak sales **multi-bn**[1]	Peak sales **multi-bn**	Peak sales **multi-bn**	Peak sales **multi-bn**
US LoE 2029+	US LoE 2025-2036	US LoE 2031+	US LoE 2031+	US LoE 2031+	US LoE 2036+

nm – not meaningful. All growth rates in constant currencies (cc). US LoEs are estimated based on relevant patents; further extensions possible. 1. Including Zolgensma® IT. 2. Including Kisqali® adjuvant.

Cosentyx® grew double digit

Sales evolution

USD m, % cc



Maintaining strong momentum

- Growing ahead of the market in rheumatology
- Steady volume growth in US / EU, acceleration in other international markets
- >700k patients across 5 indications treated worldwide since launch

Expecting double-digit growth in 2022

- China market expansion continues
- HS submissions in 2022 (~400k potential addressable patients)
- CHMP decision expected for JPsA / ERA in Q2 2022

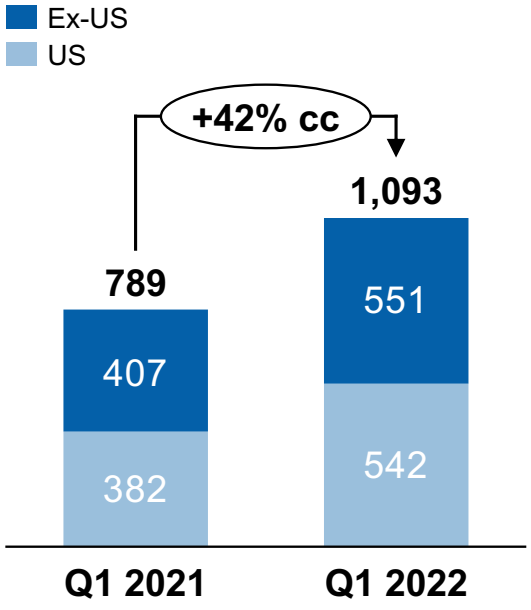
Confirming expectations of USD 7bn+ peak sales

HS – Hidradenitis Suppurativa JPsA – Juvenile Psoriatic Arthritis ERA – Enthesitis related arthritis

Entresto® +42% cc, growing strongly across geographies

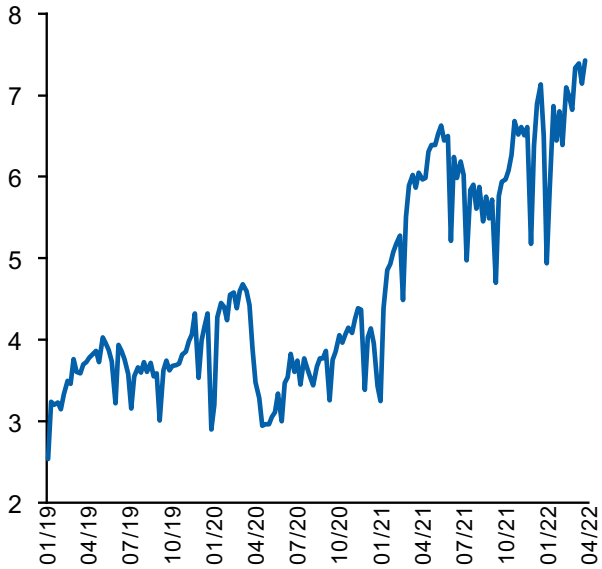
Sales evolution

USD m, % cc



US weekly NBRx[1]

New-to-brand prescriptions (000)



Strong quarter performance[2]

- US: growth across hospitals, cardiology and primary care
- Europe: strong demand growth
- China: NRDL-driven growth in HF/ HTN

Confident in future growth

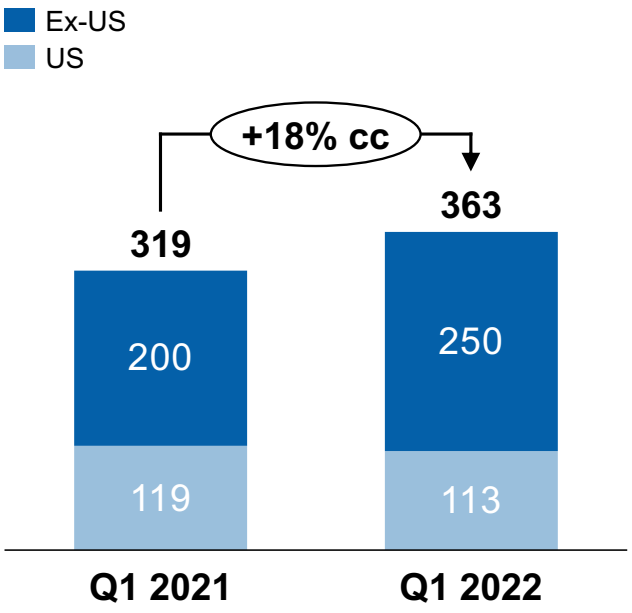
- Broad evidence across clinical and real-world settings[3,4]
- Guidelines drive 1st choice in HFrEF[5]
- Opportunity for further penetration in HF and uptake in HTN in China/ Japan[2]

NBRx – New-to-brand Prescriptions NRDL – National Reimbursement Drug List HF – Heart Failure HTN – Hypertension See References at end of this presentation

Zolgensma® grew 18% cc with increasing access ex-US

Sales evolution

USD m, % cc



Q1 highlights

- Ex-US sales grew +32% cc, while US sales steady
- Over 2000 patients have been treated worldwide

Future growth drivers

- Increase in newborn screening: currently at 95% in US, 25% in EU
- OAV101 IT data[1]: STEER currently enrolling; STRENGTH to start in 2H22

New data at MDA 2022 reinforce Zolgensma IV clinical benefit

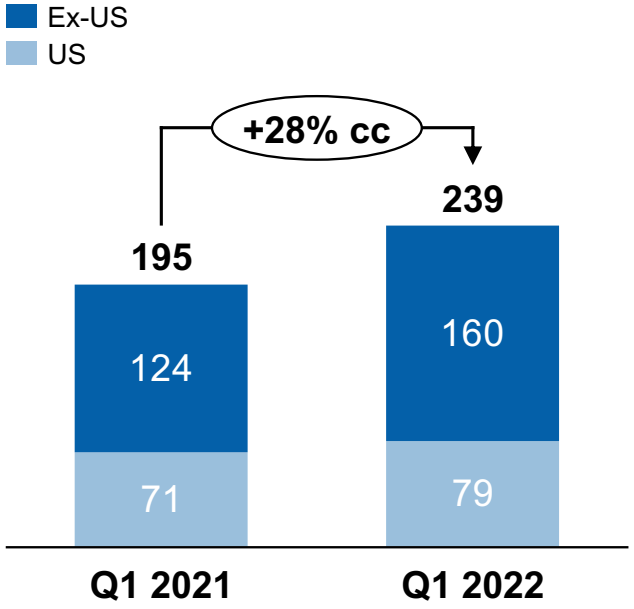
- Age-appropriate motor milestones in pre-symptomatic children with 3-copy SMN-2 backup gene (SPR1NT)
- Post-hoc analysis (START and STR1VE) of children with Type 1 SMA achieved / maintained important measures of bulbar function

1. With investigational OAV101 intrathecal administration

Kisqali® delivers double-digit growth across regions

Sales evolution

USD m, % cc



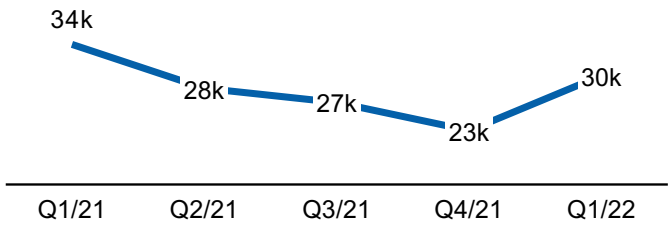
- CDK4/6 (TRx) market trending towards recovery to pre-COVID levels; US growth mainly driven by adjuvant use
- Kisqali® grew 28% cc vs. PY; US growing in line with market, Europe growing ahead of market
- MONALEESA-2 results published in NEJM, showing ~5 years median overall survival, longest ever reported in aBC
- NATALEE adjuvant study primary analysis now expected 2023

In ph3 randomized controlled trials, ribociclib + endocrine therapy has shown overall survival benefit in the first-line setting.

Strong Kesimpta® launch in a suppressed market*

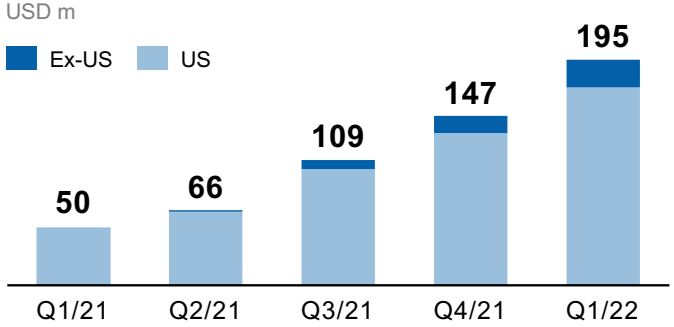
US MS market* growth still suppressed

NBRx



Sales evolution

USD m



Accelerating launch momentum

- About 20k patients treated, >60% naive or first switch[4]
- US: strong growth despite suppressed market dynamics
- Ex-US: approved in 68 countries

Strengthening differentiation and benefit/ risk profile

- Up to 4 years data show continued reduced risk of disability worsening and stable IgG levels (ASCLEPIOS / ALITHIOS)[1,2]
- Majority of Kesimpta treated patients can mount a robust immune response after COVID-19 vaccination; may be distinct amongst B cell treatments[3]

*Refers to dynamic market. NBRx – New to brand Prescription IgG – Immunoglobulin G mRNA – messenger Ribonucleic Acid. See References at end of this presentation.

Leqvio® US launch in line with expectations

Driving broad customer engagement

>90%	of prioritized HCPs reached[1]
>2x	increase in unaided awareness[2]
2 doses*	a year seen as key differentiator[2]
DTC	initiated

Establishing access and acquisition pathways

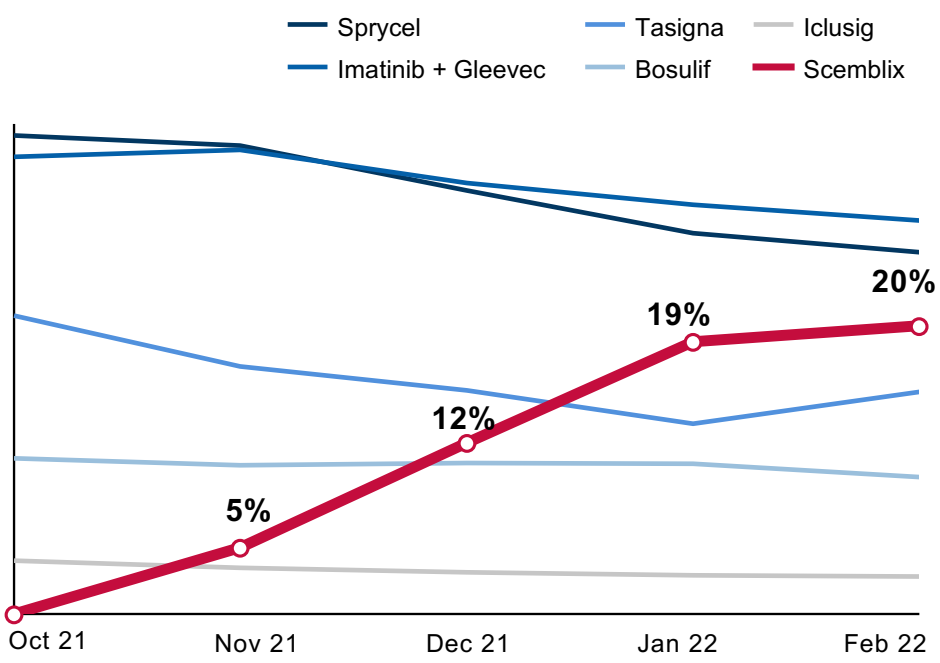
>35	of ~200 prioritized systems have ordered Leqvio[3]
~55%	of corporate AIC accounts have purchased Leqvio[3]
>30%	of customers have placed repeat orders[3]
~50%	lives have coverage aligned to label[4]

Permanent J-code (J1306) has been granted and will go into effect July 1, providing greater reimbursement confidence

HCP – Healthcare Professional AIC – Alternative Injection Center 1. Internal tracking. Data on file 2. Compared to Q4 2021. Based on market research. Data on file 3. Based on sales data, Data on file. 4. Internal tracking of Formal medical policies published to date and early reimbursement experience. Data on file. *LEQVIO® is administered initially, again at 3 months, and then once every 6 months.

Scemblix® US launch off to a strong start

NBRx share (rolling 3 months)



Strong launch execution

$25m	Uptake driven by patients with resistance/intolerance to other TKIs
20%	NBRx share in CML (across lines)
49%	3L+ patient share
85%	FF calls F2F, supporting strong SOV
1L	Ph3 study enrolling ahead of plan

FF – Field force SOV – Share of voice

Positive leading indicators for Pluvicto™ US launch

Strong benefit/risk profile reflected in label

- ☑ Population: PSMA+ mCRPC patients post ARPI and taxane[1]
- ☑ Patient selection: Using Locametz® or other approved ^{68}Ga-PSMA-11 agent[1]
- ☑ Clinical benefit: 38% reduction in risk of death vs. SOC alone[1]
- ☑ Administration: Up to 6 infusions, 6 weeks apart[1]

US launch building on Lutathera® experience

- ☑ Commercial field teams trained on promotional materials <5 days
- ☑ High awareness among ~240 target RLT treatment centers (existing Lutathera® sites)
- ☑ 96% of RLT centers have product purchase agreements in place
- ☑ >40 RLT centers onboarded to ordering system; first patients infused in April
- ☑ CMS applications submitted for permanent A code, expected to be effective in October

EU approval anticipated in H2 2022. Additional Ph3 studies ongoing in pre-taxane and hormone sensitive settings, with potential to expand patient population for Pluvicto™ 3-4x. Evaluating additional Ph3 studies.

1. Pluvicto [prescribing information]. Millburn, NJ: Advanced Accelerator Applications USA, Inc.; 2022.

Sandoz business dynamics continue to normalize, benefitting from a lower prior year comparison

SANDOZ

Sandoz stabilizing

Q1 sales USD 2.4 bn (+8%) driven by Europe (+9%)

- Retail USD 1.8 bn (+11%)
- Biopharma USD 0.5 bn (+7%)
- Benefitting from low PY comparator

Core Operating Income USD 538m (+26%)
Benefitting from low PY comp (due to cough & cold season)

Assumptions for FY
Continuing geopolitical uncertainty, price erosion, inflationary pressures

Solid base for strong mid-term growth

Driven by biosimilars, significant LOE opportunity
Targeting USD 80bn originator sales (2030)

Critical success factors on track

- ☑ Leading biosimilars pipeline: 15+ assets
- ☑ Manufacturing scale and expertise
- ☑ Development and regulatory experience
- ☑ Global footprint
- ☑ Experience in commercialization
  Leading in Europe; expanding US, RoW

Strategic review of Sandoz continues to progress, update expected at latest by end 2022

All % growth relate to cc unless otherwise stated

Broad pipeline of novel medicines continued to progress in Q1

Neuroscience | Cardio-Renal | Oncology | Immunology

Approvals

✓ **Pluvicto®**	US: mCRPC post-taxane
✓ **Vijoice®[1]**	US: PROS
✓ **Scemblix®**	JP: CML 3L
✓ **Beovu®**	EU: DME
✓ **Tafinlar+Mekinist**	CN: NSCLC with BRAF V600 mutation

Submissions

✓ **Tislelizumab**	EU: NSCLC, 2L ESCC
✓ **Aimovig®**	CN: Migraine

Readouts and publications

✓ **Tislelizumab**	Ph3 – Gastric cancer (primary OS endpoint)
✓ **JDQ443**	Encouraging early data (Ph1b KontRASt-01)

Designations

✓ **Iptacopan**	China Breakthrough in C3G
✓ **Tislelizumab**	US Orphan in 1L nasopharyngeal cancer
✓ **Scemblix®**	JP priority review in CML 3L

Major study starts

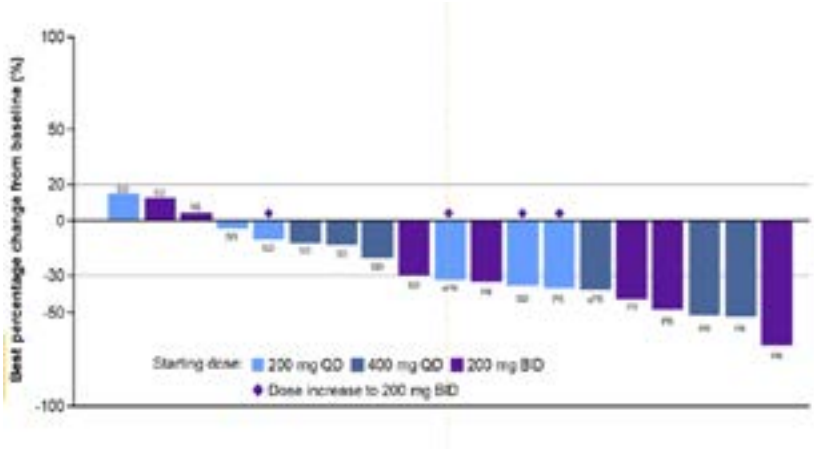
✓ **T-Charge™** PHE885	Ph2 in Multiple Myeloma
✓ **Zolgensma®**	Ph3 – SMA IT (STEER)

1. Post quarter event Selected milestones

JDQ443 showed early sign of clinical activity with acceptable safety and tolerability

KontRASt-01: Phase 1b/2 study of JDQ443 in advanced, KRAS G12C-mutated solid tumors

Best ORR across all doses in NSCLC



JDQ443, a novel KRAS G12Ci, demonstrated a competitive safety and efficacy profile in NSCLC

- ORR of **57%** (4/7) at the RD of 200 mg BID
- ORR of **45%** (9/20) across all dose levels
- No Grade 3 or higher treatment-related AEs at RD

JDQ443 achieved high systemic exposure

- PK/PD modelling predicted sustained, high-level target occupancy at the RD

Further development ongoing

- KontRASt-01 actively recruiting into SHP2i (TNO155) and anti-PD1 (tislelizumab) combo cohorts
- KontRASt-02 Ph3 JDQ443 monotherapy vs docetaxel in NSCLC opening soon

Data presented at American Association for Cancer Research ORR – Objective response rate RD – Recommended dose BID – Orally twice daily

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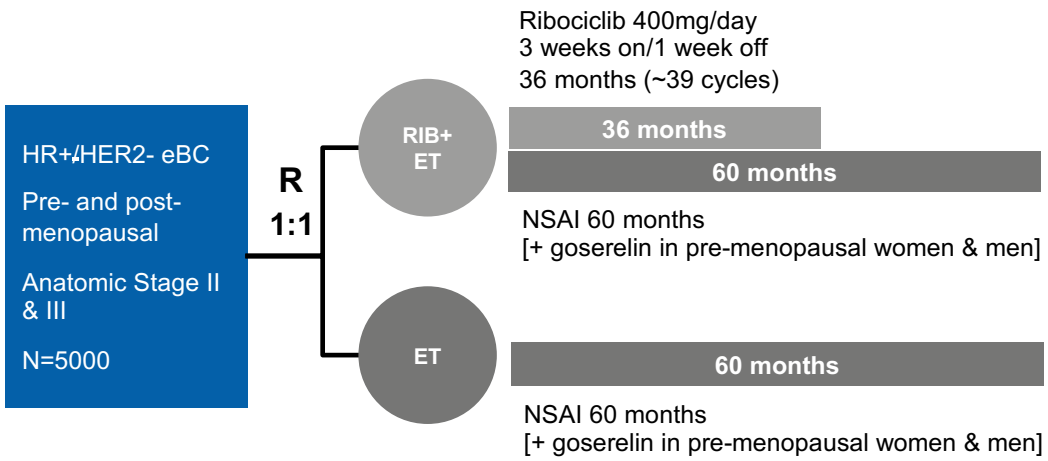
NATALEE: Latest event rate forecast indicates readout now in 2023, as event rate lower than originally projected

NATALEE study design



- Recruitment completed to 5,000 patients, including intermediate and high risk (Apr 2021)
- Primary analysis planned at 500 iDFS events; ~300 events to date
- Successfully completed futility analysis in Q1 2022
- Two interim analyses for efficacy planned; none has occurred yet
- Discontinuation rate remains low

eBC – early breast cancer 1. Projections based on data up to 03-Mar-2022. As of 20-Apr-2022, 303 events have been documented.

2022 events[1] (expected)

NME Lead

✓ Achieved ✓ Readout not supportive ✕ Missed

Regulatory decisions	H1	Pluvicto™ mCRPC (US ✓ /EU)
	H1	Vijoice® PROS (US ✓)
	H2	Scemblix® 3L CML (JP ✓ /EU)
	H2	tislelizumab ESCC 2L (US)
	H1/H2	Jakavi® acute & chronic GVHD (EU /JP)
	H1/H2	Kymriah® r/r follicular lymphoma (US/EU/JP)
	H1/H2	Beovu® DME (US/EU ✓/JP)
Submissions	H1	ensovibep COVID-19 (US ✓)
	H1/H2	Cosentyx® HS (EU/US)
	H1/H2	tislelizumab NSCLC (EU ✓ /US)
	H2	tislelizumab 1L Nasopharyngeal cancer (US)
	H2	Cosentyx® Psoriatic Arthritis IV (US)
Submissions-enabling readouts	H2	canakinumab NSCLC Ph3 Canopy A
	H2	iptacopan PNH Ph3 APPLY-PNH
	H2	Kisqali® HR+/HER2- BC (adj) Ph3 NATALEE (event driven now moving to 2023)
	H2	lutetium (177Lu) vipivotide tetraxetan mCRPC[1], pre-taxane Ph3 PSMAfore[2]

Other readouts	H1	sabatolimab HR-MDS Ph2
	H1	Cosentyx® Lichen planus Ph2 PRELUDE
	H1	Cosentyx® axSpA IV Ph3 INVIGORATE-1
	H1	icenticaftor COPD Ph2b
	H2	UNR844 presbyopia Ph2 READER
Ph3/pivotal study starts	H1	Cosentyx® peripheral SpA
	H1	OAV101 SMA IT STEER ✓
	H1	ensovibep COVID-19 (EMPATHY Part B)
	H2	JDQ443 NSCLC mono
	H2	ianalumab Sjögren's Syndrome
	H2	ianalumab Lupus Nephritis
	H2	ociperlimab solid tumors
	H2	Pluvicto™ nmCRPC
	H2	YTB323 2L DLBCL
	H2	OAV101 SMA IT Ph3b STRENGTH

1. Selected. 2. Could move to early 2023.

Harry Kirsch

Chief Financial Officer

Financial review and 2022 guidance

Q1 mid single-digit sales and high single-digit Core OpInc growth

Group[1] USD million	Q1 2022	Change vs. PY % USD	 %cc
Net sales	**12,531**	1	5
Core operating income	**4,083**	3	9
Operating income	**2,852**	18	26
Net income	**2,219**	8	15
Growth ex. prior year Roche income		*23*	*32*
Core EPS (USD)	**1.46**	-4	2
Growth ex. prior year Roche income		*6*	*12*
EPS (USD)	**1.00**	10	17
Growth ex. prior year Roche income		*25*	*34*
Free cash flow	**920**	-42	
Growth ex. prior year Roche dividend		*-14*	

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 35 of the Condensed Interim Financial Report. A reconciliation of 2021 IFRS results and non-IFRS measures core results and free cash flow to exclude the impacts of the 2021 divestment of our Roche investment can be found on page 40 of the Condensed Interim Financial Report. The free cash flow impact represents the dividend received in Q1 2021 from Roche in relation to the distribution of its 2020 net income.

Q1 Group core margin increased by 110bps to 32.6%

driven by Sandoz which benefitted from low PY base

	Q1 2022			
	Net sales change vs PY[1]	**Core operating income** change vs. PY[1]	**Core margin**[1]	**Core margin** change vs. PY[1]
	% cc	% cc	%	%pts cc
Innovative Medicines	4	5	35.9	0.2
Sandoz	8	26	22.8	3.3
Group	**5**	**9**	**32.6**	**1.1**

1. Constant currencies (cc), core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 35 of the Condensed Interim Financial Report .

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2022 Novartis full year guidance

Barring unforeseen events; growth vs. PY in cc

Innovative Medicines	Sales expected to **grow mid single digit** Core OpInc expected to **grow mid to high single digit, ahead of sales**
Sandoz	Sales expected to be **broadly in line with prior year** Core OpInc expected to **decline low to mid single digit**
Group	**Sales expected to grow mid single digit** **Core OpInc expected to grow mid single digit**

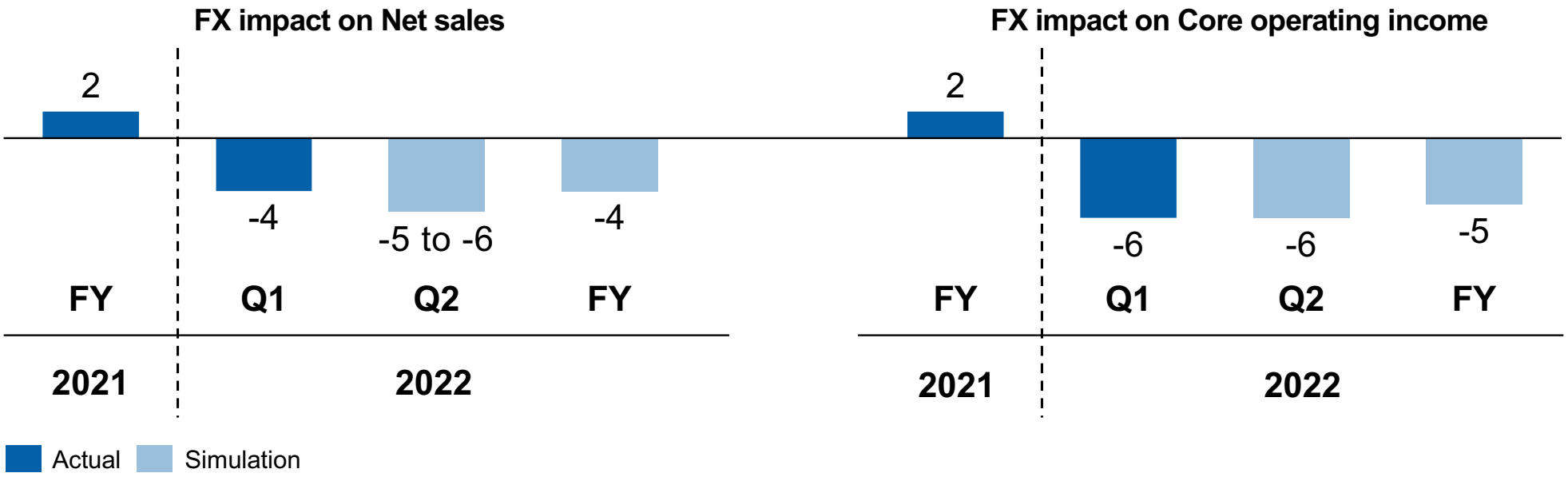
Key assumptions

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

Expected currency impact for full year 2022

Currency impact vs. PY

%pts, assuming late-April exchange rates prevail in 2022



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Vas Narasimhan

Chief Executive Officer

Novartis appoints Aharon (Ronny) Gal, Ph.D. as Chief Strategy & Growth Officer

- Effective no later than August 1, 2022
- Leads the newly created Strategy & Growth function that combines corporate strategy, R&D portfolio strategy and business development
- Reports to CEO and joins Executive Committee of Novartis
- Brings over 20 years of life-sciences industry experience including financial research and analytics, management consulting and business development

Top 2022 priorities for Novartis on track

1 **Successful launches:** Leqvio®, Kesimpta®, Pluvicto™, Scemblix®

2 **Maintain growth momentum:** 

3 **Progress pipeline**: 20+ assets with significant sales potential, approval by 2026, on track

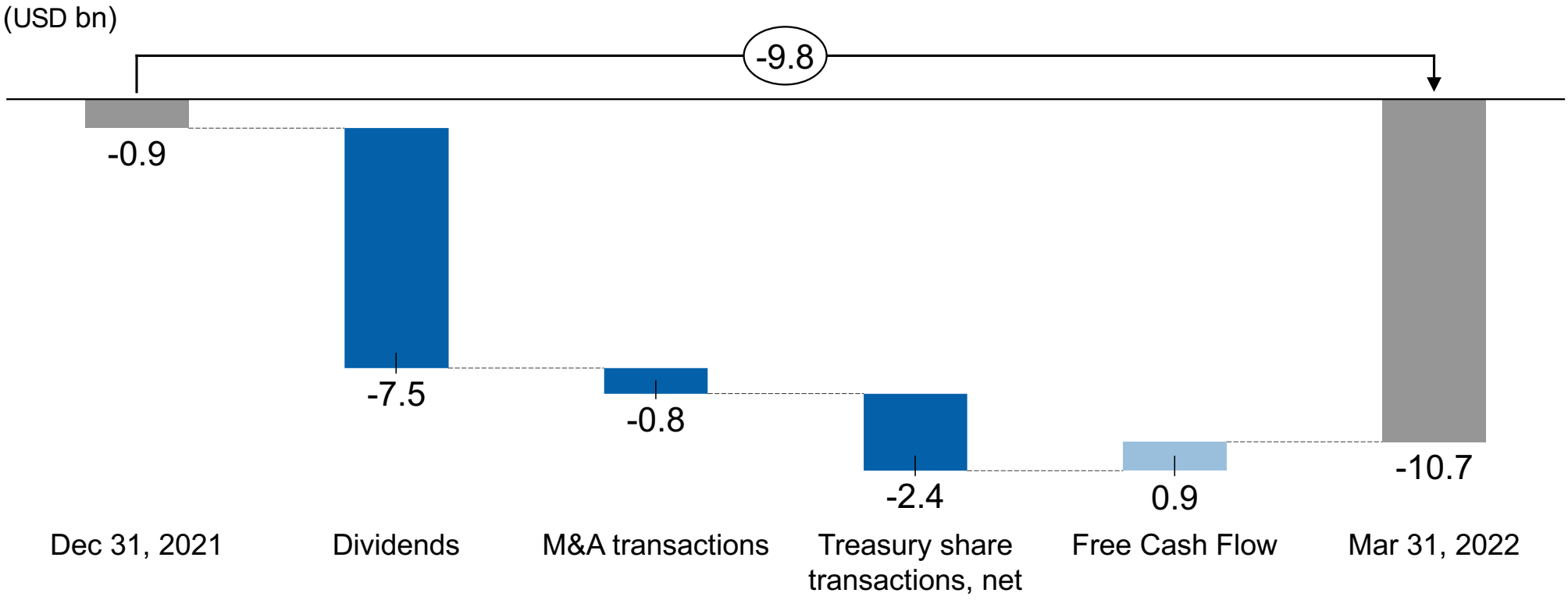
4 **Optimize portfolio:** Sandoz review, update end 2022; disciplined business development

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

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

Appendix

Net debt increased by USD 9.8bn mainly due to the annual dividend payment and share buybacks



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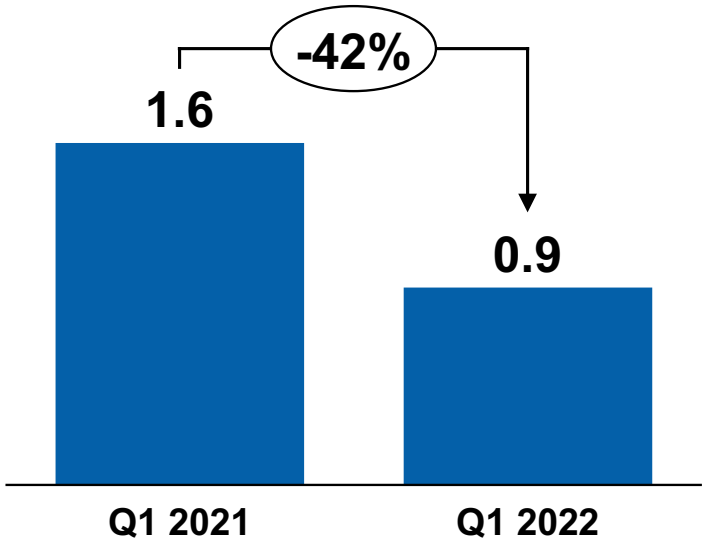
Q1 2022 free cash flow decreased to USD 0.9bn mainly due to the loss of the Roche dividend in the prior year quarter

Group free cash flow[1]

USD bn, % USD



Key drivers vs. PY

- − Lower dividends from associated companies (PY Roche cash inflow of USD 0.5bn)
- − Unfavorable working capital
- \+ Favorable hedging results

Excluding PY Roche annual dividend, free cash flow declined -14% (USD)

1. Free cash flow is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 35 of the Condensed Interim Financial Report.

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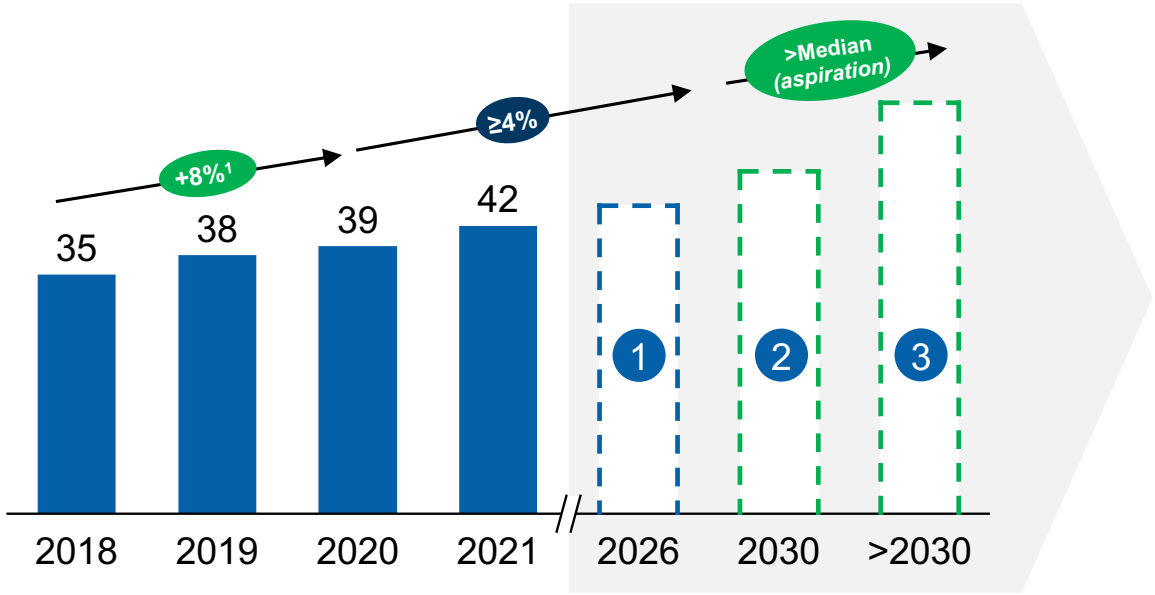
Novartis is committed to driving consistent growth through 2030 and beyond

IM sales evolution

Illustrative, USD bn, % CAGR cc



1. **2020-2026 | ≥4%**
   Focused resources on key growth brands and launches, upscaling next generation engagement models

2. **2026-2030 | >peer median**
   Double-down on internal pipeline assets to unlock their full potential and add complementary BD&L

3. **>2030 | >peer median**
   Focused investments in technology platforms while staying at the forefront of innovation in small and large molecules

1. 6% in USD.

NOVARTIS | Reimagining Medicine

Confident in future growth driven by our strength and depth in cardio-renal, immunology, neuroscience...

Selected assets, nearly all with exclusivity into 2030+

New for Q1

Cardio-Renal

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

Neuroscience

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

Immunology

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Cosentyx®	HS		Ph3 SUNRISE, SUNSHINE positive readout	2022
	GCA	●●●	Ph3 ongoing	2024
	Lupus Nephritis		Ph3 SELUNE ongoing	2026+
	Lichen Planus		Ph2b PRELUDE readout in 2022	2025
Ligelizumab	Food allergy[3]	●●●	Ph3 ongoing	2025
Remibrutinib[1]	CSU	●●●	Ph3 REMIX-1 and -2 ongoing	2024
	Other indications being explored			
Ianalumab	Sjögren's		Ph3 start in 2022	2026+
	SLE		Ph2a ongoing	2026+
	Autoimmune hepatitis	●●●	Ph2b ongoing	2026+
	Lupus Nephritis		Ph3 start in 2022	2026+
Iscalimab	Sjögren's		Ph2b ongoing	2026+
	Liver Tx	●●	Ph2b ongoing	2026+
	HS		Ph2a ongoing	2026+

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

'Wild Cards' LNA043 (osteoarthritis: Ph2 ongoing), ecleralimab (CSJ117 asthma: Ph2b ongoing, COPD: Ph2 ongoing), QBW251 (COPD: Ph2b readout imminent), SAF312 (COSP: Ph2b ongoing), UNR844 (presbyopia: Ph2b readout H2 2022)

1. Peak sales potential based on all studied indications. 2. Based on 9 months UPCR readout (US accelerated approval). 3. Food Allergy indication falls within the Respiratory & Allergy therapeutic area.

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… and strength and depth in oncology

Selected assets, nearly all with exclusivity into 2030+

New for Q1

Solid Tumors

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Kisqali®	HR+/HER2- BC (adj)	●●●	Ph3 NATALEE readout event-driven, expected 2023	2023
Canakinumab	NSCLC adjuvant	●●	Ph3 CANOPY-A readout in 2022	2023
Pluvicto®	mCRPC post-taxane	●●●	US approved	-
	mCRPC pre-taxane		Ph3 PSMAfore readout event-driven, end 2022[1]	2023
	mHSPC		Ph3 PSMAddition ongoing	2024
JDQ443 KRAS inhibitor	2/3L NSCLC (mono)	●●●	Ph3 start in H2 2022	2024
	NSCLC (combo)		Ph2 ongoing	2026+
TNO155 SHP2 inhibitor	Solid tumors: multiple combinations being explored in ongoing trials			
Tislelizumab[2]	2L esophageal cancer	●●	Submitted in EU	-
	NSCLC		Submitted in EU	-
			H2 2022 2L US submission	
	Other indications		Ongoing trials	-
Ociperlimab[2] TIGIT mab	NSCLC	●●●	Ph3 ongoing[4]	
	Other indications		Ongoing trials[4]; additional Ph3 study initiation H2 2022	

Hematology

Asset	Indication	Peak Sales	Next Milestone/ Status	Submission
Scemblix® (asciminib)	CML 3L	●●●	US and JP approved	-
	CML 1L		Ph3 ongoing	2025
Iptacopan[2]	PNH	●●●	Readout in 2022 (APPLY-PNH – enrollment completed)	2023
	aHUS		Ph3 ongoing	2025
Sabatolimab	HR-MDS	●●●	Ph2 STIMULUS-MDS-1 continues to PFS readout[3]	2022/2023
			Ph3 STIMULUS-MDS-2 ongoing	
	AML		Ph2 STIMULUS-AML-1 ongoing	2024
YTB323 CD19 CAR-T	Non-Hodgkin's Lymphoma	●●●	Ph3 start 2022	2025
PHE885 BCMA CART-T	Multiple myeloma	●	Ph2 initiated	2024

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

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

1. Could move to early 2023. 2. Peak sales potential based on all studied indications; Novartis territories. 3. Planned DMC readout for CR completed, study continues blinded to PFS readout, with submission in 2022/2023 using PFS and/or OS outcomes of Ph2 and/or Ph3 trial. 4. Active trials are being conducted by BeiGene, option deal.

NOVARTIS | Reimagining Medicine

20+ potential billion USD+ pipeline assets with approval by 2026

Most are supported by high strength of evidence

Selected assets	Strength of evidence **Moderate**	Strength of evidence **High**		
Unprobabilized peak sales **USD bn / multi-bn**	**Sabatolimab** MDS; AML	**Iptacopan** PNH; C3G; IgAN; aHUS	**Kisqali** Adj. BC (+endocrine th.) ◆	**Leqvio** Hypercholesterolemia ✓
	NIS793 PDAC; Colorectal Cancer	**Remibrutinib** CSU; MS	**YTB323**[2] 2L DLBCL	**Cosentyx** Multiple indications ◆
	Pelacarsen CVRR	**Zolgensma** SMA IT ◆	**Ianalumab** Sjogren's; SLE; AIH; Lupus Nephritis	**Pluvicto** ✓ mCRPC post-taxane; mCRPC pre-taxane; mHSPC
	Canakinumab Adj. NSCLC	**Ligelizumab** FA	**Ensovibep** Coronavirus infection	**Scemblix** ✓ 3L+ CML; 1L CML
	Ociperlimab[1] NSCLC	**Ociperlimab** NSCLC		**Tislelizumab** Multiple indications
	UNR844 Presbyopia			**Piqray (alpelisib)** ✓ PROS; HER2+ adv BC; TNBC; ovarian cancer ◆
	Libvatrep (SAF312) Chronic Ocular Surface Pain			
	TNO155, JDQ443[2] NSCLC; Colorectal Cancer; Combos			
Unprobabilized peak sales **up to USD 1bn**		**Lutathera** 1L G2/G3 NET ◆	**Kymriah** r/r Follicular Lymphoma ◆	**Beovu** DME ✓ ◆
			Tafinlar/Mekinist Solid Tumor Agnostic ◆	**Jakavi** SR GvHD ◆

Most advanced and key indication(s) **approved by 2026**

- Submission
- Phase III
- Phase II
- ◆ LCM
- ✓ Approved

Strength of evidence based on the most advanced indication: High if in Ph3 or when Ph2 results available for the same MoA in the lead indication.

1. BeiGene option deal. 2. Ph3 to start in 2022. Assets are shown in the phase of the most advanced indication (listed first). Value based on the total of the listed indication(s).

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Key milestones of pipeline assets with significant sales potential with approval by 2026

Selected assets, most advanced and key indication(s) approved by 2026

High strength of evidence	2022	2023	2024	2025	2026
Iptacopan PNH	Ph3 readout	Submission			
Iptacopan C3G		Ph3 read/sub			
Iptacopan aHUS			Ph3 readout	Submission	
Iptacopan IgAn		Ph3 read/sub			
Remibrutinib CSU			Ph3 read/sub		
Remibrutinib MS				Ph3 read/sub	
Zolgensma SMA IT			Ph3 readout	Submission	
Ligelizumab Food Allergy				Ph3 read/sub	
Kisqali		Ph3 read/sub			
YTB323 2L DLBCL	Ph3 start			Ph3 read/sub	
Ianalumab Sjögren's	Ph3 start				Ph3 read/sub
Ianalumab LN	Ph3 start				Ph3 read/sub
Cosentyx HS	Submission				
Cosentyx Lichen Planus	Ph2 readout			Submission	
Cosentyx GCA			Ph3 read/sub		
Pluvicto mCRPCR post taxane	Approved				
Pluvicto mCRPCR pre taxane	Ph3 readout[1]	Submission			
Pluvicto tetraxetan mHSPC			Ph3 read/sub		

NME Lead

High strength of evidence	2022	2023	2024	2025	2026
Ensovibep Coronavirus infection	Submission				
Scemblix	Approval				
Scemblix CML 1L			Ph3 readout	Submission	
Vijoice PROS	Approval				
Piqray Ovarian Cancer		Ph3 read/sub			
Piqray TNBC		Ph3 read/sub			
Piqray HER2+ adv BC				Ph3 read/sub	
Tislelizumab	Submissions and approvals of several indications				

Moderate strength of evidence	2022	2023	2024	2025	2026
Sabatolimab MDS	Ph2 readout	Ph3 readout	*Submission in 2022/23*		
Sabatolimab AML		Ph3 readout	Submission		
NIS793 PDAC				Ph3 read/sub	
Pelacarsen CVRR				Ph3 read/sub	
Canakinumab Adj. NSCLC	Ph3 readout	Submission			
Ociperlimab	Ph3 start	*BeiGene option deal*			
UNR844 Presbyopia	Ph2 readout		Submission		
Libvatrep COSP		Ph2 readout			Submission
JDQ443 NSCLC	Ph3 start		Submission		
JDQ443+TNO155 NSCLC		Ph3 start			Submission

1. Event driven, could move to early 2023.

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Our pipeline projects at a glance

	Phase 1/2	Phase 3	Registration	Total
Innovative medicines	104	50	6	160
Solid Tumors	25	20	2	47
Hematology	20	7	3	30
Immunology	26	7	0	33
Neuroscience	6	5	0	11
Cardiovascular, Renal, Metabolism	5	6	0	11
Others	22	5	1	28
Ophthalmology	*6*	*1*	*0*	*7*
Respiratory & Allergy	*8*	*3*	*0*	*11*
Global Health	*8*	*1*	*1*	*10*
Biosimilars	0	2	0	2
Total	**104**	**52**	**6**	**162**

NOVARTIS | Reimagining Medicine

Novartis pipeline in Phase 1

28 lead indications

Lead indication

Solid tumors

Code	Name	Mechanism	Indication(s)
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Multiple solid tumors
AAA817	Ac-PSMA-617	Radioligand therapy target PSMA	Metastatic castration-resistant prostate cancer
ADPT01	ADPT01	-	Colorectal cancer (combos)
DFF332	DFF332	HIF2A inhibitor	Renal cell carcinoma
DKY709	DKY709 + spartalizumab	Novel immunomodulatory agent	Cancers
IAG933	IAG933	-	Mesothelioma
JDQ443	JDQ443	KRAS Inhibitor	KRAS G12C mutated solid tumors
KAZ954	KAZ954	-	Solid tumors
NIS793	NIS793, spartalizumab	TGFB1 inhibitor	Solid tumors
NIZ985	NIZ985, spartalizumab	IL-15 agonist	Solid tumors
NZV930	NZV930, spartalizumab, NIR178	CD73 antagonist	Solid tumors
PDR001	spartalizumab	PD1 inhibitor	Solid tumors (combo)
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 + MBG453, venetoclax	MDM2 inhibitor	Haematological malignancy
JBH492	JBH492	-	Haematological malignancy
JEZ567	JEZ567	CD123 CAR-T	Acute myeloid leukaemia
MAK683	MAK683	EED inhibitor	Cancers
MBG453	sabatolimab	TIM3 antagonist	Low risk myelodysplastic syndrome
MIK665	MIK665	MCL1 inhibitor	Acute myeloid leukaemia (combo)
VAY736	ianalumab + ibrutinib	BAFF-R inhibitor	Haematological malignancy
VOB560	VOB560	-	Cancers
WVT078	WVT078	-	Multiple myeloma
YTB323	YTB323	CD19 CAR-T	DLBCL and adult ALL

Others

Code	Name	Mechanism	Indication(s)
Global Health			
EYU688	EYU688	NS4B inhibitor	Dengue
KAF156	ganaplacide	-	Malaria prophylaxis
INE963	INE963	-	Malaria, uncomplicated
Respiratory & Allergy			
LTP001	LTP001	-	Respiratory diseases
NCJ424	NCJ424	-	Respiratory diseases
Ophtalmology			
MHU650	MHU650	-	Diabetic eye diseases

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Novartis pipeline in Phase 2

30 lead indications

Lead indication

Solid Tumors

Code	Name	Mechanism	Indication(s)
AAA601	Lutathera®	Radioligand therapy target SSTR	GEPNET, pediatrics
			1L ES-SCLC
			Glioblastoma
BLZ945	sotuletinib	CSF-1R inhibitor	Solid tumors
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	HGG/LGG, pediatrics
INC280	Tabrecta®	Met inhibitor	Non-small cell lung cancer (Combo)
JDQ443	JDQ443	KRAS inhibitor	NSCLC (combo)
NIR178	NIR178, spartalizumab	Ad2AR inhibitor, PD1 inhibitor	Cancers
NIS793	NIS793	TGFB1 inhibitor	1L metastatic colorectal cancer
TNO155	TNO155	SHP2 inhibitor	Solid tumors (single agent)

Immunology

Code	Name	Mechanism	Indication(s)		
AIN457	Cosentyx®	IL17A inhibitor	Lichen planus		
CFZ533	iscalimab	CD40 inhibitor	Sjögren's	Liver Tx	Hidradenitis suppurativa
CMK389	CMK389	IL-18 inhibitor	Atopic dermatitis		
DFV890	DFV890	NLRP3 inhibitor	Osteoarthritis		
			Familial cold auto-inflammatory syndrome		
LJN452	tropifexor + licogliflozin	FXR agonist	Non-alcoholic steatohepatitis (Combos)		
LNA043	LNA043	ANGPTL3 agonist	Knee osteoarthritis	Osteoarthritis (combos)	
LOU064	remibrutinib	BTK inhibitor	Sjögren's		
LRX712	LRX712	-	Osteoarthritis		
LYS006	LYS006	Anti-inflammatory	Acne	Colitis ulcerative	Hidradenitis suppurativa
MAS825	MAS825	-	NLRC4-GOF indications	Hidradenitis suppurativa	
MHV370	MHV370	-	Sjögren's	Mixed connective tissue disease	
VAY736	ianalumab	BAFF-R inhibitor	Sjögren's	Autoimmune hepatitis	
			Systemic lupus erythematosus		

Neuroscience

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

Hematology

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

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)	
CFZ533	iscalimab	CD40 inhibitor	Lupus nephritis	Type 1 diabetes mellitus
HSY244	HSY244	-	Atrial fibrillation	
LNP023	iptacopan	CFB inhibitor	Membranous nephropathy	
MBL949	MBL949	-	Obesity related diseases	

Others

Code	Name	Mechanism	Indication(s)	
Global Health				
KAE609	cipargamin	PfATP4 inhibitor	Malaria, severe	Malaria, uncomplicated
KAF156	ganaplacide	-	Malaria, uncomplicated	
LXE408	LXE408	Proteasome inhibitor	Visceral leishmaniasis	
SEG101	Adakveo®	P-selectin inhibitor	Sickle cell anaemia with crisis, pediatrics	
Respiratory & Allergy				
CMK389	CMK389	IL-18 inhibitor	Pulmonary sarcoidosis	
CSJ117	ecleralimab	TSLP inhibitor	Asthma	Chronic obstructive pulmonary disease
QBW251	icenticaftor	CFTR potentiator	Chronic obstructive pulmonary disease	Bronchiectasis
QMF149	Atectura®	Combo	Asthma, pediatrics	
Ophtalmology				
CPK850	CPK850	RLBP1 AAV	Retinitis pigmentosa	
LKA651	LKA651	EPO inhibitor	Diabetic retinopathy	
PPY988	PPY988	Gene therapy	Geographic atrophy	
SAF312	libvatrep	TRPV1 antagonist	Chronic ocular surface pain	
UNR844	UNR844	Reduction of disulfide bonds	Presbyopia	

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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)		
ACZ885	canakinumab	IL-1b inhibitor	NSCLC, adjuvant		
BYL719	Piqray®	PI3Kα inhibitor	HER2+ adv BC	Triple negative breast cancer	Ovarian cancer
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	Thyroid cancer		
INC280	Tabrecta®	Met inhibitor	Non-small cell lung cancer		
JDQ443	JDQ443	KRAS inhibitor	2/3L Non-small cell lung cancer		
LEE011	Kisqali®	CDK4 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	Hidradenitis suppurativa
			Psoriatic arthritis (IV formulation)	
			Axial SpA (IV formulation)	
			Giant cell arteritis	
LOU064	remibrutinib	BTK inhibitor	Chronic spontaneous urticaria	
VAY736	ianalumab	BAFF-R inhibitor	Lupus Nephritis[3]	

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
CTL019	Kymriah®	CD19 CAR-T	1L high risk acute lymphocytic leukaemia, pediatrics & young adults
ETB115	Promacta®	Thrombopoietin receptor (TPO-R) agonist	Radiation sickness syndrome
LNP023	iptacopan	CFB inhibitor	Paroxysmal nocturnal haemoglobinuria
			Atypical haemolytic uraemic syndrome
MBG453	sabatolimab	TIM3 antagonist	Myelodysplastic syndrome
YTB323	YTB323	CD19 CAR-T	2L Diffuse large B-cell lymphoma[3]

Cardiovascular, Renal, Metabolism

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

Others

Code	Name	Mechanism	Indication(s)	
Global Health				
COA566	Coartem®	-	Malaria, uncomplicated (<5kg patients)	
Respiratory & Allergy				
IGE025	Xolair®	IgE inhibitor	Food allergy	Auto-injector
QGE031	ligelizumab	IgE inhibitor	Food allergy	
Ophthalmology				
RTH258	Beovu®	VEGF inhibitor	Diabetic retinopathy	

Biosimilars

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

1. ^{177}Lu-dotatate in US. 2. Approved in US. 3. Ph3 to be initiated in 2022.

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Novartis pipeline in registration

2 lead indications

Lead indication

Solid Tumors

Code	Name	Mechanism	Indication(s)
VDT482	tislelizumab	PD1 inhibitor	2L ESCC
			Non-small cell lung cancer

Hematology

Code	Name	Mechanism	Indication(s)
CTL019	Kymriah®	CD19 CAR-T	r/r Follicular lymphoma
INC424	Jakavi®	JAK1/2 inhibitor	Acute GVHD
			Chronic GVHD

Others

Code	Name	Mechanism	Indication(s)
Global Health			
SKO136	ensovibep	Multi-specific DARPin	Corona virus infection

NOVARTIS | Reimagining Medicine

Novartis submission schedule

New Molecular Entities: Lead and supplementary indications

LEAD INDICATIONS

2022

- **sabatolimab**[1] — Lead — MBG453, HR-MDS
- **ensovibep** — Lead — SKO136, COVID19

2023

- **iptacopan** — Lead — LNP023, PNH

2024

- **JDQ443** — Lead — JDQ443, 2/3L NSCLC (mono)
- **remibrutinib** — Lead — LOU064, CSU
- **UNR844** — Lead — Presbyopia

2025

- **icenticaftor** — Lead — QBW251, COPD
- **ligelizumab** — Lead — QGE031, Food allergy
- **NIS793** — Lead — 1L Pancreatic cancer
- **pelacarsen** — Lead — TQJ230, CVRR-Lp(a)
- **YTB323** — Lead — 2L Diffuse large B-cell lymphoma

≥2026

- **^{177}Lu-NeoB** — Lead — AAA603, Multiple Solid Tumors
- **branaplam** — Lead — LMI070, Huntington's disease
- **cipargamin** — Lead — KAE609, Malaria severe
- **CPK850** — Lead — RP
- **ecleralimab** — Lead — CSJ117, Asthma
- **gevokizumab** — Lead — VPM087, 1st line CRC
- **ganaplacide** — Lead — KAF156, Malaria uncomplicated
- **iscalimab** — Lead — CFZ533, Sjögren's syndrome
- **ianalumab** — Lead — VAY736, Sjögren's syndrome
- **libvatrep** — Lead — SAF312, COSP
- **LNA043** — Lead — Knee osteoarthritis
- **LXE408** — Lead — Visceral leishmaniasis
- **MIJ821** — Lead — Acute depression
- **PPY988** — Lead — Geographic atrophy
- **TNO155** — Lead — Solid tumors
- **tropifexor&licogliflozi** — Lead — LJN452, NASH (combos)

NEW INDICATIONS

2022

- **tislelizumab** — LCM — VDT482, 1L Nasopharyngeal Carcinoma
- **tislelizumab** — LCM — VDT482, NSCLC

2023

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

2024

- **Pluvicto** — LCM — AAA617, mHSPC
- **sabatolimab** — LCM — MBG453, Unfit AML
- **tislelizumab** — LCM — VDT482, 1L Small Cell Lung Cancer

2025

- **Scemblix** — LCM — ABL001, CML 1L
- **iptacopan** — LCM — LNP023, aHUS
- **remibrutinib** — LCM — LOU064, Multiple sclerosis

≥2026

- **Scemblix** — LCM — ABL001, CML, 2L, pediatrics
- **cipargamin** — LCM — KAE609, Malaria uncomplicated
- **JDQ443** — LCM — JDQ443, NSCLC (combo)
- **ianalumab** — LCM — VAY736, AIH
- **ianalumab** — LCM — VAY736, Lupus Nephritis
- **ianalumab** — LCM — VAY736, SLE
- **iscalimab** — LCM — CFZ533, Liver Tx
- **iptacopan** — LCM — LNP023, iMN
- **remibrutinib** — LCM — LOU064, Sjögren's syndrome
- **tislelizumab** — LCM — VDT482, Adj/Neo adj NSCLC
- **tislelizumab** — LCM — VDT482, 1L Urothelial Cell Carcinoma

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

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Novartis submission schedule

Supplementary indications for existing brands

2022

Cosentyx LCM
secukinumab, AIN457
PsA IV

Cosentyx LCM
secukinumab, AIN457
Hidradenitis suppurativa

Entresto EU[1] LCM
sacubitril/valsartan, LCZ696
Pediatric CHF

Tafinlar + Mekinist LCM
dabrafenib + trametinib, DRB436
HGG/LGG - Pediatrics

Xolair LCM
omalizumab, IGE025
Auto-injector

2023

canakinumab LCM
ACZ885
Adjuvant NSCLC

Cosentyx LCM
secukinumab, AIN457
axSpA IV

denosumab BioS
GP2411
anti RANKL mAb

Kisqali **LCM**
ribociclib, LEE011
HR+/HER2- BC (adj)

Lutathera LCM
^{177}Lu-oxodotreotide[2]
GEP-NET 1L G3

Piqray LCM
alpelisib, BYL719
TNBC

Piqray LCM
alpelisib, BYL719
Ovarian cancer

Xolair LCM
omalizumab, IGE025
Food allergy

2024

Adakveo LCM
SEG101
Sickle cell anaemia with crisis ped

Coartem LCM
artemether + lumefantrine, COA566
Malaria uncompl., formula for <5kg

Cosentyx LCM
secukinumab, AIN457
GCA

Jakavi LCM
ruxolitinib, INC424
Pediatrics Acute GVHD

Jakavi LCM
ruxolitinib, INC424
Pediatrics Chronic GVHD

Tafinlar + Mekinist LCM
dabrafenib + trametinib, DRB436
Thyroid cancer

2025

aflibercept BioS
SOK583
Neovascular age-related macular degeneration

Beovu LCM
brolucizumab, RTH258
Diabetic retinopathy

Cosentyx LCM
secukinumab, AIN457
Lichen Planus

Leqvio LCM
KJX839
Ped Hyoerlipidemia

Piqray LCM
alpelisib, BYL719
HER2+ adv BC

Promacta LCM
eltrombopag, ETB115
Radiation sickness syndrome

Zolgensma LCM
AVXS-101 OAV101
SMA IT

≥2026

Atectura LCM
indacaterol + mometasone, QMF149
Asthma, pediatrics

Aimovig LCM
erenumab, AMG334
Pediatric Migraine

Cosentyx LCM
secukinumab, AIN457
Lupus Nephritis

Jakavi LCM
ruxolitinib, INC424
Myelofibrosis (combination)

Kesimpta[3] LCM
ofatumumab
Multiple sclerosis, pediatrics

Kymriah LCM
tisagenlecleucel, CTL019
1L high risk ALL, pediatrics & young adults

Leqvio LCM
KJX839
CVRR-LDLC

Mayzent[4] LCM
siponimod, BAF312
Multiple sclerosis, pediatrics

Rydapt LCM
midostaurin, PKC412
Acute myeloid leukemia, pediatrics

Tabrecta LCM
capmatinib, INC280
NSCLC

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

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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

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Cardiovascular, Renal and Metabolic

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Entresto® - Angiotensin receptor/neprilysin inhibitor

Study	NCT02678312 PANORAMA HF (CLCZ696B2319)	NCT02884206 PERSPECTIVE (CLCZ696B2320)
Indication	Heart failure in pediatric patients	Heart failure
Phase	Phase 3	Phase 3
Patients	377	592
Primary Outcome Measures	Part 1: Pharmacodynamics and pharmacokinetics of sacubitril/valsartan LCZ696 analytes Part 2: Efficacy and safety compared with enalapril	Change from baseline in the CogState Global Cognitive Composite Score (GCCS)
Arms Intervention	Part 1: Sacubitril/valsartan 0.8 mg/kg or 3.1 mg/kg or both; 0.4 mg/kg or 1.6 mg/kg or both (single doses). Part 2: enalapril/placebo 0.2 mg/kg bid (ped. formulation 1mg/ml) and adult formulation (2.5, 5, 10 mg bid); Sacubitril/valsartan (LCZ696)/placebo: Ped. formulation granules (12.5, 31.25 mg in capsules); liquid formulation (1mg/ml and 4mg/ml concentration) and adult formulation (50, 100, 200 mg bid)	Sacubitril/valsartan 50, 100, and 200 mg bid with placebo of valsartan Valsartan 40, 80, and 160 mg bid tablets with placebo for sacubitril/valsartan
Target Patients	Pediatric patients from 1 month to < 18 years of age with heart failure due to systemic left ventricle systolic dysfunction	Patients with chronic heart failure with preserved ejection fraction
Read-out Milestone(s)	2022; (Analysis of 110 pts from Part 2 formed the basis for pediatric submission in Apr-2019 and approval by the US FDA in Oct-2019 for the treatment of symptomatic HF with systemic left ventricular systolic dysfunction in children aged 1 year and older)	2023
Publication	TBD	TBD

NOVARTIS | Reimagining Medicine

Entresto® - Angiotensin receptor/neprilysin inhibitor

Study	NCT03785405 (CLCZ696B2319E1 - extension study)
Indication	Heart failure in pediatric patients
Phase	Phase 3
Patients	240
Primary Outcome Measures	Number of participants with Adverse Events (AEs) and Serious Adverse Events (SAEs)
Arms Intervention	Single arm, open label sacubitril/valsartan (pediatric formulation granules (12.5, 31.25 mg in capsules); liquid formulation (1mg/ml and 4mg/ml concentration) and adult formulation (50, 100, 200 mg bid))
Target Patients	Pediatric patients with heart failure due to systemic left ventricle systolic dysfunction who have completed study CLCZ696B2319
Read-out Milestone(s)	2023
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	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 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.
Target Patients	Patient population with mean baseline LDL-C >= 100mg/dL
Read-out Milestone(s)	2026
Publication	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	NCT03060577 ORION-3 (CKJX839A12201E1)	NCT03814187 ORION-8 (CKJX839A12305B)
Indication	Hypercholesterolemia inc. Atherosclerotic Cardiovascular Disease (ASCVD) and ASCVD risk equivalents Heterozygous Familial Hypercholesterolaemia (HeFH)	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH) and Homozygous Familial Hypercholesterolemia (HoFH)
Phase	Phase 2	Phase 3
Patients	490	2991
Primary Outcome Measures	LDL-C reduction at Day 210 for Group 1 subjects Changes in other lipids and lipoproteins and reduction of LDL-C of more than 50% for patients that are above LDL-C goal ; longer term exposure and safety.	Proportion of subjects achieving pre specified low density lipoprotein cholesterol (LDL-C) targets at end of study Safety and tolerability profile of long term use of inclisiran
Arms Intervention	Group 1 - inclisiran sodium 300mg sc on Day 1 and every 180 days thereafter for up to 4 years. Group 2- Evolocumab 140mg s.c. injection on Day 1 and every 2 weeks until Day 336, followed by inclisiran sodium 300mg on Day 360, Day 450 and then every 6 months for a planned duration of 4 years.	Inclisiran sodium 300mg on day 1 (placebo patients entered into study from ORION 9, 10 & 11) or placebo on Day 1 (inclisiran patients entered into study from ORION 9, 10 & 11) then inclisiran sodium 300mg on Day 90 and every 6 months for a planned duration of 3 years
Target Patients	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy and risk equivalents (patients from ORION 3, 9, 10 & 11 studies)
Read-out Milestone(s)	H1-2022	2023
Publication	TBD	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	NCT03851705 ORION-5 (CKJX839A12302)	NCT04652726 ORION-16 (CKJX839C12301)
Indication	Hypercholesterolemia inc. Homozygous Familial Hypercholesterolemia (HoFH)	Hyperlipidemia, pediatrics
Phase	Phase 3	Phase 3
Patients	56	150
Primary Outcome Measures	LDL-C reduction at Day 150 Changes in PCSK9, other lipids and lipoproteins	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to Day 330
Arms Intervention	Part 1: inclisiran sodium 300mg on Day 1 and Day 90 or placebo on Day 1 and Day 90 Part 2: inclisiran sodium 300mg on Day 180 for patients who were randomized to the placebo group only, inclisiran sodium 300mg on Day 270 and then every 6 months for a planned duration of 2 years for all patients	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	Patients with HoFH with background statin +/- ezetimibe therapy	Adolescents (12 to less than 18 years) with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C)
Read-out Milestone(s)	Primary: Q3-2020 (actual); Final: H2-2021	2025
Publication	TBD	TBD

Leqvio® - siRNA (regulation of LDL-C)

Study	NCT04659863 ORION-13 (CKJX839C12302)	NCT05030428 VICTORION-2P (CKJX839B12302)
Indication	Hyperlipidemia, pediatrics	CVRR
Phase	Phase 3	Phase 3
Patients	15	15000
Primary Outcome Measures	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to day 330	1. Time to First Occurrence of 3P-MACE (3-Point Major Adverse Cardiovascular Events)
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.	Arm 1: Experimental Inclisiran sodium, Subcutaneous injection Arm 2: Placebo Comparator, Placebo Subcutaneous injection
Target Patients	Adolescents (12 to less than 18 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDL-C)	Participants with established cardiovascular disease (CVD)
Read-out Milestone(s)	2025	2027
Publication	TBD	TBD

iptacopan - CFB inhibitor

Study	NCT04817618 APPEAR-C3G (CLNP023B12301)	NCT03955445 (CLNP023B12001B)
Indication	C3 glomerulopathy	C3 glomerulopathy (C3G)
Phase	Phase 3	Phase 2
Patients	68	27
Primary Outcome Measures	Log-transformed ratio to baseline in UPCR (sampled from a 24 hour urine collection)	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	Experimental: iptacopan 200mg b.i.d. Placebo Comparator: Placebo to iptacopan 200mg b.i.d.	Open-label LNP023 200mg bid
Target Patients	Patients with native C3G	Patients with C3 glomerulopathy
Read-out Milestone(s)	2023	2025
Publication	TBD	Wong et al 2021 Nephrology, Dialysis and Transplantation Vol. 36, Suppl. 1: eGFR trajectory

iptacopan - CFB inhibitor

Study	NCT04154787 (CLNP023D12201)	NCT04578834 APPLAUSE-IgAN (CLNP023A2301)
Indication	Idiopathic membranous nephropathy (iMN)	IgA nephropathy
Phase	Phase 2	Phase 3
Patients	72	450
Primary Outcome Measures	Change from baseline of UPCR derived from 24hr urine collections at Baseline and Week 24	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	LNP023 low dose LNP023 high dose Rituximab	Arm 1 - LNP023 200mg BID Arm 2 - Placebo BID
Target Patients	Patients with biopsy proven iMN who are at high risk of disease progression defined on the basis of antibody anti-PLA2R titre and proteinuria	Primary IgA Nephropathy patients
Read-out Milestone(s)	2023	2023 (primary endpoint for US initial submission, 9 months UPCR)2025 (24 months)
Publication	TBD	Perkovic et al. 2021, Nephrology Dialysis Transplantation, Vol. 36, Suppl. 1: Study Design Wong et al. 2021, Nephrology Dialysis Transplantation, Vol. 36, Suppl. 1: IPTACOPAN (LNP023): A NOVEL ORAL COMPLEMENT ALTERNATIVE PATHWAY FACTOR B INHIBITOR SAFELY AND EFFECTIVELY STABILISES EGFR IN C3 GLOMERULOPATHY

pelacarsen - ASO targeting Lp(a)

Study	NCT04023552 Lp(a)HORIZON (CTQJ230A12301)
Indication	Cardiovascular risk reduction
Phase	Phase 3
Patients	7680
Primary Outcome Measures	Time to the first occurrence of MACE (cardiovascular death, non-fatal MI, non-fatal stroke and urgent coronary re-vascularization)
Arms Intervention	TQJ230 80 mg injected monthly subcutaneously or matched placebo
Target Patients	Patients with a history of Myocardial infarction or Ischemic Stroke, or a clinically significant symptomatic Peripheral Artery Disease, and Lp(a) >= 70 mg/dL
Read-out Milestone(s)	2025
Publication	TBD

Immunology

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LNA043- ANGPTL3 agonist

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

Cosentyx® - IL-17A inhibitor

Study	NCT03031782 (CAIN457F2304)	NCT03259074 SURPASS (CAIN457K2340)
Indication	JPsA & ERA	JPsA & ERA
Phase	Phase 3	Phase 3
Patients	80	837
Primary Outcome Measures	Time to 33 flares	No radiographic structural progression as measured by modified Stoke Ankylosing Spondylitis Spine Score (mSASSS)
Arms Intervention	Secukinumab (pre-filled syringe) 75 mg Placebo	Secukinumab 150/300 mg Adalimumab biosimilar 40 mg
Target Patients	Juvenile idiopathic arthritis subtypes of psoriatic and enthesitis-related arthritis	Patients with active ankylosing spondylitis
Read-out Milestone(s)	H1-2021	2022
Publication	H2-2021	Study design manuscript published. Baraliakos et al. Clinical Drug Investigation (2020) 40:269-278.

Cosentyx® - IL-17A inhibitor

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

Cosentyx® - IL-17A inhibitor

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

Cosentyx® - IL-17A inhibitor

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

Cosentyx® - IL-17A inhibitor

Study	NCT04209205 INVIGORATE-2 (CAIN457P12302)	NCT04300296 PRELUDE (CAIN457S12201)
Indication	Psoriatic Arthritis (PsA)	Lichen Planus
Phase	Phase 3	Phase 2
Patients	380	108
Primary Outcome Measures	The proportion of subjects achieving American College of Rheumatology 50 (ACR50) response criteria	Proportion of patients achieving Investigator's Global Assessment (IGA 0/1) score at 16 weeks +30% delta vs placebo
Arms Intervention	Secukinumab intravenous (i.v.) regimen Placebo intravenous (i.v.) regimen	Secukinumab 300 mg s.c. Placebo s.c.
Target Patients	Patients with active psoriatic arthritis (PsA) despite current or previous NSAID, DMARD and/or anti-TNF therapy	Adult patients with biopsy-proven lichen planus not adequately controlled by topical therapies
Read-out Milestone(s)	H2-2021 (Actual)	2022
Publication	2023	TBD

Cosentyx® - IL-17A inhibitor

Study	NCT04930094 (CAIN457R12301)
Indication	Giant cell arteritis
Phase	Phase 3
Patients	240
Primary Outcome Measures	Number of participants with sustained remission
Arms Intervention	Experimental: Secukinumab 300 mg Placebo Comparator: Placebo
Target Patients	Patients with Giant Cell Arteritis (GCA)
Read-out Milestone(s)	Primary 2024 Final 2025
Publication	TBD

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ianalumab - BAFF-R inhibitor

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

iscalimab - CD40 inhibitor

Study	NCT03781414 CONTRAIL I (CCFZ533A2202)	NCT03905525 TWINSS (CCFZ533B2201)
Indication	Liver transplantation	Sjögren's syndrome
Phase	Phase 2	Phase 2
Patients	128	260
Primary Outcome Measures	Proportion of patients with composite event (BPAR, Graft Loss or Death) over 12 months	Change in EULAR Sjögren's syndrome Disease Activity Index (ESSDAI) score and EULAR Sjögren's syndrome Patient Reported Index (ESSPRI) score
Arms Intervention	Control/Standard of Care: TAC + MMF + Corticosteroids CFZ533 dose A + MMF + Corticosteroids CFZ533 dose B + MMF + Corticosteroids	Three dose arms of CFZ533 Placebo
Target Patients	Liver transplant recipients	Patients with Sjögren's syndrome
Read-out Milestone(s)	2023	2022
Publication	2023	2022

iscalimab - CD40 inhibitor

Study	**NCT04541589 TWINSS Extn (CFZ533B2201E1)**
Indication	Sjögren's syndrome
Phase	Phase 2
Patients	
Primary Outcome Measures	Incidence of Treatment-emergent AEs (TEAEs) Change in laboratory evaluations for hematology from baseline to each study visit Change in laboratory evaluations for serum chemistry from baseline to each study visit Change in vital sign measurements from baseline for each post-baseline visit
Arms Intervention	Arm 1 - Iscalimab Dose 1 s.c. Q2W Arm 2 - Iscalimab Dose 2 s.c. Q2W and Placebo
Target Patients	Patients with Sjögren's Syndrome, who participated in the TWINSS core study, CCFZ533B2201(NCT03905525)
Read-out Milestone(s)	Primary completion date: 2024
Publication	

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remibrutinib - BTK inhibitor

Study	NCT04109313 (CLOU064A2201E1)
Indication	Chronic spontaneous urticaria (CSU)
Phase	Phase 2
Patients	250
Primary Outcome Measures	Long-term safety and tolerability
Arms Intervention	Selected dose of LOU064 taken orally twice a day (morning and evening) from day 1 to week 52
Target Patients	Patients with CSU who have participated in preceding studies with LOU064
Read-out Milestone(s)	H2 2022
Publication	Primary: 2023

remibrutinib - BTK inhibitor

Study	NCT05030311 REMIX-1 (CLOU064A2301)	NCT05032157 REMIX-2 (CLOU064A2302)
Indication	Chronic spontaneous urticaria (CSU)	Chronic spontaneous urticaria (CSU)
Phase	Phase 3	Phase 3
Patients	450	450
Primary Outcome Measures	1. Change from baseline in UAS7 (Scenario 1 with UAS7 as primary efficacy endpoint) 2. Absolute change in ISS7 and absolute change in HSS7 (Scenario 2 with ISS7 and HSS7 as co-primary efficacy endpoints)	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) 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)	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. Randomised in 2:1 ratio (active vs placebo) 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. Randomised in 2:1 ratio (active vs placebo)
Target Patients	Adult participants suffering from chronic spontaneous urticaria (CSU) inadequately controlled by H1-antihistamines in comparison to placebo	Adult participants suffering from chronic spontaneous urticaria (CSU) inadequately controlled by H1-antihistamines in comparison to placebo
Read-out Milestone(s)	2024	2024
Publication	TBD	TBD

tropifexor, licogliflozin - FXR agonist and SGLT 1/2 inhibitor

Study	NCT04065841 ELIVATE (CLJN452D12201C)
Indication	Non-alcoholic steatohepatitis (NASH)
Phase	Phase 2
Patients	380
Primary Outcome Measures	Proportion of patients with resolution of NASH and no worsening of fibrosis OR improvement in fibrosis by at least one stage without worsening of NASH at Week 48 compared with baseline
Arms Intervention	Arm A: combination therapytropifexor + licogliflozin Arm B: tropifexor monotherapytropifexor + licogliflozin placebo Arm C: licogliflozin monotherapylicogliflozin + tropifexor placebo Arm D: licogliflozin placebo + tropifexor placebo
Target Patients	Adult patients with biopsy based non-alcoholic steatohepatitis (NASH) and liver fibrosis
Read-out Milestone(s)	2024
Publication	2025

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Neuroscience

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MIJ821- NR2B negative allosteric modulator (NAM)

Study	NCT04722666 (CMIJ821A12201)
Indication	Major depressive 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 or 0.9% sodium chloride
Target Patients	Participants who have suicidal ideation with intent
Read-out Milestone(s)	2023
Publication	TBD

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Aimovig® - CGRP receptor antagonist

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

LMI070 - mRNA splicing modulator

Study	NCT05111249 VIBRANT-HD (CLMI070C12203)
Indication	Huntington`s disease
Phase	Phase 2
Patients	75
Primary Outcome Measures	1. Reduction (%) of mHTT protein in cerebrospinal fluid (CSF) 2. Number of treatment emergent adverse events and serious adverse events
Arms Intervention	Arm 1: Experimental; Branaplam 56 mg oral solution once weekly Arm 2: Experimental; Branaplam 112 mg oral solution once weekly Arm 3: Experimental; (C) Branaplam 154 mg oral solution once weekly, OR (X) Branaplam 84 mg oral solution once weekly OR (Y) Branaplam 28 mg oral solution once weekly Arm 4: Placebo; Matching placebo oral solution once weekly
Target Patients	Participants with early manifest Huntington's Disease
Read-out Milestone(s)	2025
Publication	TBD

Kesimpta® - CD20 antagonist

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

Mayzent® - S1P1,5 receptor modulator

Study	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). he targeted enrollment is 180 participants with multiple sclerosis which will include at least 5 participants with body weight (BW) ?40 kg and at least 5 participants with age 10 to 12 years in each of the ofatumumab and siponimod arms. There is a minimum 6 month follow up period for all participants (core and extension). Total duration of the study could be up to 7 years.
Read-out Milestone(s)	2026
Publication	TBD

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remibrutinib - BTK inhibitor

Study	NCT05147220 REMODEL-1 (CLOU064C12301)	NCT05156281 REMODEL-2 (CLOU064C12302)
Indication	Multiple sclerosis	Multiple sclerosis
Phase	Phase 3	Phase 3
Patients	800	800
Primary Outcome Measures	Annualized relapse rate (ARR) of confirmed relapses	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)	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	Patients with relapsing Multiple Sclerosis
Read-out Milestone(s)	Estimated primary completion 2025 Estimated study completion 2029	Estimated primary completion 2025 Estimated study completion 2029
Publication	TBD	TBD

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Zolgensma® - SMN1 gene replacement therapy

Study	NCT05089656 STEER (COAV101B12301)
Indication	Spinal muscular atrophy (IT administration)
Phase	Phase 3
Patients	125
Primary Outcome Measures	1. Change from baseline in Hammersmith functional motor scale - Expanded (HFMSE) total score at the end of follow-up period 1 in treated patients compared to sham controls in the ≥ 2 to < 18 years age group
Arms Intervention	Arm 1: Experimental OAV101. Administered as a single, one-time intrathecal dose Arm 2: Sham Comparator: Sham control. A skin prick in the lumbar region without any medication.
Target Patients	Patients Type 2 Spinal Muscular Atrophy (SMA) who are ≥ 2 to < 18 years of age, treatment naive, sitting, and never ambulatory
Read-out Milestone(s)	2024
Publication	TBD

Ophthalmology

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UNR844 - Reduction of disulfide bonds

Study	NCT04806503 READER (CUNR844A2022)
Indication	Presbyopia
Phase	Phase 2B
Patients	225
Primary Outcome Measures	Characterize the dose response relationship among UNR844 doses 0 mg/mL, 5 mg/mL, 13.3 mg/mL, 23 mg/mL and 30 mg/mL dosed twice-daily after Month 3 of dosing. Change from baseline in Binocular distance-corrected near visual acuity at 40 cm at Month 3.
Arms Intervention	1:1 randomization - UNR844 0 mg/mL, 5 mg/mL, 13.3 mg/mL, 23 mg/mL and 30 mg/mL dosed twice-daily for three months
Target Patients	Presbyopic participants aged 45 to 55 years
Read-out Milestone(s)	2022: Primary endpoint- when all patients have completed the 3 months treatment period 2023: Final analysis -Study completion (all patients have completed 9 months pots treatment period)
Publication	H1-2023

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Beovu® - Anti-VEGF

Study	NCT04005352 TALON (CRTH258A2303)
Indication	Neovascular Age-related Macular Degeneration (nAMD)
Phase	Phase 3B
Patients	739
Primary Outcome Measures	Average change in Best-corrected visual acuity Distribution of the last interval with no disease activity (in a Treat-to-Control regimen)
Arms Intervention	Arm 1: Brolucizumab 6 mg intravitreal injection Arm 2: Aflibercept 2 mg intravitreal injection
Target Patients	Patients with Neovascular Age-related Macular Degeneration (nAMD) who have not previously received anti-VEGF (vascular endothelial growth factor) treatment
Read-out Milestone(s)	2022
Publication	TBD

Beovu® - Anti-VEGF

Study	NCT04047472 HOBBY (CRTH258A2307)
Indication	Macular degeneration
Phase	Phase 3
Patients	494
Primary Outcome Measures	Change from baseline in best-corrected visual acuity (BCVA) at week 48
Arms Intervention	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL
Target Patients	Chinese patients with neovascular age-related macular degeneration
Read-out Milestone(s)	2024
Publication	TBD

Beovu® - VEGF Inhibitor

Study	NCT03917472 KINGFISHER (CRTH258B2305)	NCT04058067 KINGLET (CRTH258B2304)
Indication	Diabetic macular edema	Diabetic macular edema
Phase	Phase 3	Phase 3
Patients	500	263
Primary Outcome Measures	Change in best-corrected visual acuity (BCVA) from baseline up to week 52	Change in best-corrected visual acuity (BCVA)
Arms Intervention	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL	Brolucizumab (RTH258) 6 mg/50 µL Aflibercept 2 mg/50 µL
Target Patients	Patients with visual impairment due to diabetic macular edema	Chinese patients with visual impairment due to diabetic macular edema
Read-out Milestone(s)	Q3-2021 (Actual)	2023
Publication	Publication submission planned for H1-2022	Publication planned for 2024

Beovu® - VEGF Inhibitor

Study	NCT04278417 (CRTH258D2301)
Indication	Diabetic retinopathy
Phase	Phase 3
Patients	706
Primary Outcome Measures	Change from Baseline in BCVA
Arms Intervention	Arm1: RTH258 (brolucizumab) 6 mg/50uL Arm2: Panretinal photocoagulation laser initial treatment followed with additional PRP treatment as needed
Target Patients	Patients with proliferative diabetic retinopathy
Read-out Milestone(s)	2024
Publication	TBD

libvatrep - TRPV1 antagonist

Study	NCT04630158 SAHARA (CSAF312B12201)
Indication	Chronic ocular surface pain
Phase	Phase 2
Patients	150
Primary Outcome Measures	Change in mean pain severity Visual Analog Scale
Arms Intervention	Placebo Comparator: SAF312 Placebo. Randomized to a 1:1:1 topical eye drops, twice daily Experimental: SAF312 dose 1. Randomized to a 1:1:1 topical eye drops, twice daily Experimental: SAF312 dose 2. Randomized to a 1:1:1 topical eye drops, twice daily
Target Patients	Subjects with CICP persisting at least for 4 months after refractive surgery and chronicity confirmed during the observational period.
Read-out Milestone(s)	2023
Publication	2023

Respiratory & Allergy

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CSJ117 - Inhaled TSLP inhibitor

Study	NCT04410523 (CCSJ117A12201C)
Indication	Asthma
Phase	Phase 2
Patients	625
Primary Outcome Measures	Pre-dose FEV1 (Forced Expiratory Volume in 1 second) change from baseline after 12 weeks of treatment. Average change from baseline in pre-dose FEV1 at week 8 & week 12
Arms Intervention	CSJ117 0.5mg CSJ117 1mg CSJ117 2 mg CSJ117 4 mg CSJ117 8 mg Placebo
Target Patients	Asthma patients on background medium or high ICS plus LABA therapy
Read-out Milestone(s)	2023
Publication	2023

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icenticaftor - CFTR potentiator

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

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ligelizumab - IgE inhibitor

Study	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: Experimental ligelizumab 240 mg subcutaneous injection for 52 weeks Arm 2: Experimental ligelizumab 120 mg subcutaneous injection for 52 weeks Arm 3: Experimental Placebo 8 weeks and ligelizumab 120 mg Arm 4: Placebo subcutaneous injection for first 8 weeks and ligelizumab 120 mg subcutaneous injection for 44 weeks Arm 5: Experimental Placebo 16 weeks and ligelizumab 120 mg/240 mg subcutaneous injection for 36 weeks Arm 6: Experimental Placebo 8 weeks and ligelizumab 240 mg subcutaneous injection for 44 weeks
Target Patients	Participants with a medically confirmed diagnosis of IgE-mediated peanut allergy
Read-out Milestone(s)	2025
Publication	TBD

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Oncology: Solid Tumors

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Vijoyce® - PI3K-alpha inhibitor

Study	NCT04589650 EPIK-P2 (CBYL719F12201)
Indication	PIK3CA-related overgrowth spectrum
Phase	Phase 2
Patients	174
Primary Outcome Measures	Proportion of participants with a response at Week 24
Arms Intervention	Arm 1: alpelisib vs. Arm 2: placebo during the 16 first weeks, for each cohort (adult, pediatric), with placebo patients switching to alpelisib thereafter.
Target Patients	Pediatric and adult participants with PIK3CA-related overgrowth spectrum (PROS)
Read-out Milestone(s)	Primary Analysis: 2023
Publication	NA

canakinumab - IL-1beta inhibitor

Study	NCT03631199 CANOPY-1 (CACZ885U2301)
Indication	1st Line Non-small cell lung cancer (NSCLC)
Phase	Phase 3
Patients	627
Primary Outcome Measures	Safety run-in part: Incidence of dose limiting toxicities Double-blind, randomized, placebo-controlled part: Progression free survival (PFS) Overall survival (OS)
Arms Intervention	Canakinumab or matching placebo in combination with pembrolizumab and platinum-based doublet chemotherapy
Target Patients	Patients with: Histologically confirmed Stage IIIB, IV NSCLC with no prior systemic anticancer therapy Squamous and non-squamous NSCLC No EGFR mutation and ALK rearrangement
Read-out Milestone(s)	H2-2021
Publication	Johnson B et al. Presented at AACR-NCI-EORTC 2019 (safety run-in) Planned abstract submission to AACR 2022

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canakinumab - IL-1beta inhibitor

Study	NCT03447769 CANOPY-A (CACZ885T2301)
Indication	Adjuvant NSCLC
Phase	Phase 3
Patients	1500
Primary Outcome Measures	Disease free survival (primary), overall survival (key secondary)
Arms Intervention	Canakinumab 200mg q3w sc for 18 cycles Placebo q3w sc for 18 cycles
Target Patients	Patients with: High-risk NSCLC (AJCC/UICC v.8 stage II-IIIA and IIIB (T>5cm N2)) after complete resection and standard of care adjuvant cisplatin-based chemotherapy All histologies
Read-out Milestone(s)	2022
Publication	TBD

NIS793 - TGFβ1 inhibitor

Study	NCT04935359 (CNIS793B12301)
Indication	1L metastatic pancreatic ductal adenocarcinoma
Phase	Phase 3
Patients	490
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	Randomized portion of the study: Arm 1: NIS793+gemcitabine+nab-paclitaxel Arm 2: placebo+gemcitabine+nab-paclitaxel
Target Patients	Patients with Metastatic Pancreatic Ductal Adenocarcinoma (mPDAC), first line treatment
Read-out Milestone(s)	Primary 2025
Publication	TBD

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TNO155 - SHP2 inhibitor

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

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Pluvicto®- Radioligand therapy target PSMA

Study	NCT04689828 PSMAfore (CAAA617B12302)	NCT04720157 PSMAddition (CAAA617C12301)
Indication	Metastatic castration-resistant prostate cancer, pre-taxane	Metastatic hormone sensitive prostate cancer
Phase	Phase 3	Phase 3
Patients	450	1126
Primary Outcome Measures	Radiographic Progression Free Survival (rPFS)	Radiographic Progression Free Survival (rPFS)
Arms Intervention	Arm 1: Participants will receive 7.4 GBq (200 mCi) +/- 10% lutetium (177Lu) vipivotide tetraxetan once every 6 weeks for 6 cycles. Best supportive care, including ADT may be used Arm 2: For participants randomized to the ARDT arm, the change of ARDT treatment will be administered per the physician's orders. Best supportive care, including ADT may be used	Arm 1: lutetium (177Lu) vipivotide tetraxetan Participant will receive 7.4 GBq (+/- 10%) lutetium (177Lu) vipivotide tetraxetan , once every 6 weeks (+/- 1 week) for a planned 6 cycles, in addition to the Standard of Care (SOC); ARDT +ADT is considered as SOC and treatment will be administered per the physician's order Arm 2: For participants randomized to Standard of Care arm, ARDT +ADT is considered as SOC and treatment will be administered per the physician's order
Target Patients	mCRPC patients that were previously treated with an alternate ARDT and not exposed to a taxane-containing regimen in the CRPC or mHSPC settings	Patients with metastatic Hormone Sensitive Prostate Cancer (mHSPC)
Read-out Milestone(s)	Primary Analysis: 2022 Final Analysis: 2025	Primary Analysis: 2024
Publication	TBD	TBD

Kisqali® - CDK4/6 inhibitor

Study	NCT03701334 NATALEE (CLEE011O12301C)
Indication	Adjuvant treatment of hormone receptor (HR)-positive, HER2-negative, early breast cancer (EBC)
Phase	Phase 3
Patients	5101
Primary Outcome Measures	Invasive Disease-Free Survival for using STEEP criteria (Standardized Definitions for Efficacy End Points in adjuvant breast cancer trials)
Arms Intervention	Ribociclib + endocrine therapy Endocrine therapy
Target Patients	Pre and postmenopausal women and men with HR-positive, HER2-negative EBC, after adequate surgical resection, who are eligible for adjuvant endocrine therapy
Read-out Milestone(s)	2023
Publication	TBD

Piqray® - PI3K-alpha inhibitor

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

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Piqray® - PI3K-alpha inhibitor

Study	NCT04729387 EPIK-O (CBYL719K12301)
Indication	Ovarian Cancer
Phase	Phase 3
Patients	358
Primary Outcome Measures	Progression Free Survival (PFS) based on Blinded Independent Review Committee (BIRC) assessment using RECIST 1.1 criteria
Arms Intervention	Arm 1 Experimental: Alpelisib+olaparib: Alpelisib 200 mg orally once daily and olaparib 200 mg orally twice daily on a continuous dosing schedule Arm 2 Active Comparator: Paclitaxel or PLD. Investigator's choice of one of 2 single agent cytotoxic chemotherapies: Paclitaxel 80 mg/m2 intravenously weekly or Pegylated liposomal Doxorubicin (PLD) 40-50 mg/m2 (physician discretion) intravenously every 28 days.
Target Patients	Patients with platinum resistant or refractory high-grade serous ovarian cancer, with no germline BRCA mutation detected
Read-out Milestone(s)	2023
Publication	TBD

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Tabrecta® - MET inhibitor

Study	NCT04427072 (CINC280A2301)	NCT04816214 GEOMETRY-E (CINC280L12301)
Indication	Non-small cell lung cancer	Non-small cell lung cancer
Phase	Phase 3	Phase 3
Patients	90	245
Primary Outcome Measures	Progression free survival (PFS) per blinded independent review committee (BIRC) using RECIST v1.1	Run-in part: Incidence of dose limiting toxicities (DLTs) Randomized part: Progression free survival (PFS)
Arms Intervention	Arm 1: 400mg of capmatinib tablets administered orally twice daily Arm 2: Docetaxel 75 mg/m2 by intravenous infusion every 21 days	Arm 1: Experimental: Combination of capmatinib + osimertinib (run-in part) Arm 2: Experimental: Combination of capmatinib + osimertinib (randomized part) Arm 3: Active Comparator: platinum + pemetrexed based doublet chemotherapy
Target Patients	Previously Treated Patients With EGFR wt, ALK Negative, Locally Advanced or Metastatic (Stage IIIB/IIIC or IV) NSCLC Harboring MET Exon 14 Skipping Mutation (MET?ex14).	Adult subjects with Non-small Cell Lung cancers as second line therapy
Read-out Milestone(s)	Primary 2022 Final: 2024	Primary: 2025 Final: 2027
Publication	TBD	TBD

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Tafinlar + Mekinist® - BRAF inhibitor and MEK inhibitor

Study	NCT04940052 (CDRB436J12301)
Indication	Thyroid cancer
Phase	Phase 3
Patients	150
Primary Outcome Measures	Progression Free Survival
Arms Intervention	Arm 1: Experimental: Dabrafenib plus trametinib Participants will be treated with dabrafenib twice daily and trametinib once daily Arm 2: Placebo Comparator: Placebo dabrafenib plus placebo trametinib Participants will receive placebo dabrafenib twice daily and placebo trametinib once daily
Target Patients	Previously treated patients with locally advanced or metastatic, radio-active Iodine refractory BRAFV600E mutation-positive differentiated thyroid cancer
Read-out Milestone(s)	2024
Publication	TBD

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Tafinlar + Mekinist® - BRAF inhibitor and MEK inhibitor

Study	NCT02684058 (CDRB436G2201)
Indication	BRAFV600 mutant gliomas
Phase	Phase 2
Patients	142
Primary Outcome Measures	Objective response rate
Arms Intervention	Dabrafenib + trametinib (dose based on age and weight)
Target Patients	Children and adolescent patients with BRAF V600 mutation positive relapsed or refractory high grade glioma (HGG) or BRAF V600 mutation positive low grade glioma (LGG)
Read-out Milestone(s)	Q4 2021 (actual)
Publication	TBD

Hematology

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Adakveo® - P-selectin inhibitor

Study	NCT03814746 STAND (CSEG101A2301)
Indication	Prevention of Vaso-Occlusive Crises (VOC) in patients with Sickle Cell Disease (SCD)
Phase	Phase 3
Patients	240
Primary Outcome Measures	Rate of VOC events leading to healthcare visit
Arms Intervention	Crizanlizumab 5.0 mg/kg Crizanlizumab 7.5 mg/kg Placebo
Target Patients	Adolescent and adult SCD patients (12 years and older)
Read-out Milestone(s)	2022
Publication	TBD

Adakveo® - P-selectin inhibitor

Study	NCT03474965 SOLACE-Kids (CSEG101B2201)
Indication	Prevention of VOC in pediatric patients with SCD
Phase	Phase 2
Patients	100
Primary Outcome Measures	PK/PD and safety of SEG101 at 5 mg/kg
Arms Intervention	SEG101 (crizanlizumab) at a dose of 5 mg/kg by IV infusion ± Hydroxyurea/Hydroxycarbamide
Target Patients	Pediatric SCD patients with VOC
Read-out Milestone(s)	H2-2021 (pediatric patients >=12 year old) 2024 (pediatric patients <12 year old)
Publication	TBD

Jakavi® - JAK 1/2 inhibitor

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

Jakavi® - JAK 1/2 inhibitor

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

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Kymriah® - CD19 CAR-T

Study	NCT03570892 BELINDA (CCTL019H2301)	NCT03876769 CASSIOPEIA (CCTL019G2201J)
Indication	2nd line Diffuse large B-cell lymphoma (DLBCL)	1st line high risk acute lymphoblastic leukemia (ALL)
Phase	Phase 3	Phase 2
Patients	318	160
Primary Outcome Measures	Event-free Survival (EFS)	Disease Free Survival (DFS)
Arms Intervention	Tisagenlecleucel versus standard of care	Single-arm study of tisagenlecleucel
Target Patients	Adult patients with aggressive B-cell Non-Hodgkin Lymphoma after failure of rituximab and anthracycline- containing frontline immunochemotherapy	Pediatric and young adult patients with 1st line high risk ALL
Read-out Milestone(s)	9 Jul 2021 (actual)	2025
Publication	Bishop et al at SITC 2019 Abstract submission TBD	TBD

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Promacta® - Thrombopoetin receptor agonist

Study	NCT03025698 (CETB115E2201)	NCT03988608 (CETB115E2202)
Indication	Refractory or relapsed severe aplastic anemia	Refractory or relapsed severe aplastic anemia
Phase	Phase 2	Phase 2
Patients	51	20
Primary Outcome Measures	PK of eltrombopag at steady state in pediatric patients with SAA	Hematologic response rate rate up to 26 weeks of treatment
Arms Intervention	Eltrombopag 12.5, 25, 50, 75 mg FCT & 25 mg pFOS Arm A: relapsed/refractory SAA or recurrent AA following IST for SAA: hATG/cyclosporine + eltrombopag or cyclosporine + eltrombopag Arm B: previously untreated SAA: hATG/cyclosporine + eltrombopag	Eltrombopag 25 mg film-coated tablets
Target Patients	Pediatric patients from age 1 <18 years with relapsed/refractory SAA or recurrent AA after IST or previously untreated SAA	Chinese patients with refractory or relapsed severe aplastic anemia
Read-out Milestone(s)	Primary CSR: 2022 Final CSR: 2025	Primary CSR: 2022 Final CSR: 2025
Publication	TBD	TBD

Rydapt® - Multi-targeted kinase inhibitor

Study	NCT03591510 (CPKC412A2218)
Indication	Acute myeloid leukemia, pediatrics
Phase	Phase 2
Patients	20
Primary Outcome Measures	Occurrence of dose limiting toxicities Safety and Tolerability
Arms Intervention	Chemotherapy followed by Midostaurin
Target Patients	Newly diagnosed pediatric patients with FLT3 mutated acute myeloid leukemia (AML)
Read-out Milestone(s)	2026
Publication	TBD

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Scemblix®- BCR-ABL inhibitor

Study	NCT04971226 ASC4FIRST (CABL001J12301)
Indication	Chronic myeloid leukemia, 1st line
Phase	Phase 3
Patients	402
Primary Outcome Measures	Major Molecular Response (MMR) at week 48
Arms Intervention	Arm 1: asciminib 80 mg QD Arm 2: Investigator selected TKI including one of the below treatments: - Imatinib 400 mg QD - Nilotinib 300 mg BID - Dasatinib 100 mg QD - Bosutinib 400 mg QD
Target Patients	Patients with newly diagnosed philadelphia chromosome positive chronic myelogenous leukemia in chronic phase
Read-out Milestone(s)	2024
Publication	TBD

iptacopan - CFB inhibitor - HEM

Study	NCT04558918 APPLY-PNH (CLNP023C12302)	NCT04820530 APPOINT-PNH (CLNP023C12301)
Indication	Paroxysmal nocturnal haemoglobinuria	Paroxysmal nocturnal haemoglobinuria
Phase	Phase 3	Phase 3
Patients	91	40
Primary Outcome Measures	Percentage of participants achieving a sustained increase in hemoglobin levels of >= 2 g/dL in the absence of red blood cell transfusions Percentage of participants achieving sustained hemoglobin levels >= 12 g/dL in the absence of red blood cell transfusions	Proportion of participants achieving a sustained increase from baseline in hemoglobin levels of ? 2 g/dL assessed , in the absence of red blood cell transfusions
Arms Intervention	Arm 1: Drug: LNP023, taken orally b.i.d. dosage supplied: 200 mg dosage form: hard gelatin capsule Route of Administration: Oral Arm 2: Drug: Eculizumab, administered as intravenous infusion every 2 weeks as per the stable regimen, the maintenance dose is a fixed dose. Dosage supplied: 300 mg/30mL Dosage form: Concentrate solution for infusion Drug: Ravulizumab, administered as intravenous infusion every 8 weeks, the maintenance dose is based on body weight. Dosage Supplied: 300 mg/30mL Dosage f	Iptacopan (LNP023), taken orally b.i.d. (dosage supplied: 200mg)
Target Patients	Adult patients with PNH and residual anemia, despite treatment with an intravenous Anti-C5 antibody	PNH patients who are naive to complement inhibitor therapy, including anti-C5 antibody
Read-out Milestone(s)	Primary 2022	2023
Publication	Risitano AM, et al. Abstract accepted at the European Hematology Association (EHA 2021) congress (study design abstract; accepted for publication only)	Peffault de Latour R, et al. Abstract accepted at the European Hematology Association (EHA 2021) congress (study design abstract; accepted for publication only)

iptacopan - CFB inhibitor

Study	NCT04889430 APPELHUS (CLNP023F12301)
Indication	Atypical haemolytic uraemic syndrome
Phase	Phase 3
Patients	50
Primary Outcome Measures	Percentage of participants with complete TMA response without the use of PE/PI and anti-C5 antibody
Arms Intervention	Single arm open-label with 50 adult patients receiving 200mg oral twice daily doses of iptacopan
Target Patients	Adult patients with aHUS who are treatment naive to complement inhibitor therapy (including anti-C5 antibody)
Read-out Milestone(s)	2024
Publication	TBD

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sabatolimab - TIM3 antagonist

Study	NCT03946670 STIMULUS MDS-1 (CMBG453B12201)	NCT04150029 STIMULUS-AML1 (CMBG453C12201)
Indication	Myelodysplastic syndrome	Unfit acute myeloid leukaemia
Phase	Phase 2	Phase 2
Patients	120	86
Primary Outcome Measures	Complete Remission (CR) rate and Progression Free Survival (PFS)	Incidence of dose limiting toxicities (Safety run-in patients only) Percentage of subjects achieving complete remission (CR)
Arms Intervention	Experimental: Sabatolimab (MBG453) + hypomethylating agents Placebo comparator: Placebo + hypomethylating agents	Single arm safety and efficacy study of sabatolimab in combination with azacitidine and venetoclax
Target Patients	Adult subjects with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as per IPSS-R criteria	Newly diagnosed adult AML patients who are not suitable for treatment with intensive chemotherapy
Read-out Milestone(s)	2022-2023	2023
Publication	TBD	TBD

sabatolimab - TIM3 antagonist

Study	NCT04266301 STIMULUS-MDS2 (CMBG453B12301)
Indication	Myelodysplastic syndrome
Phase	Phase 3
Patients	500
Primary Outcome Measures	Overall survival
Arms Intervention	Sabatolimab 800 mg + azacitidine 75 mg/m2 Sabatolimab 800 mg + azacitidine 75 mg/m2 + placebo
Target Patients	Patients with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as Per IPSS-R, or Chronic Myelomonocytic Leukemia-2 (CMML-2)
Read-out Milestone(s)	2023
Publication	TBD

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Biosimilars

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aflibercept - VEGF inhibitor

Study	NCT04864834 Mylight (CSOK583A12301)
Indication	Aflibercept BioS
Phase	Phase 3
Patients	460
Primary Outcome Measures	Best-corrected visual acuity (BCVA) will be assessed using the ETDRS testing charts at an initial distance of 4 meters. The change from baseline in BCVA in letters is defined as difference between BCVA score between week 8 and baseline
Arms Intervention	Arm 1 Biological: SOK583A1 (40 mg/mL) Arm 2 Biological: Eylea EU (40 mg/mL)
Target Patients	Patients with neovascular age-related macular degeneration
Read-out Milestone(s)	2023
Publication	tbd

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denosumab - anti RANKL mAb

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

Global Health

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artemether + lumefantrine

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

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ganaplacide - Imidazolopiperazines derivative

Study	NCT03167242 (CKAF156A2202)	NCT04546633 KALUMI (CKAF156A2203)
Indication	Malaria	Malaria, uncomplicated
Phase	Phase 2	Phase 2
Patients		
Primary Outcome Measures	PCR-corrected adequate clinical and parasitological response (ACPR)	PCR-corrected and uncorrected Adequate Clinical and Parasitological Response (ACPR)
Arms Intervention	KAF156 and LUM-SDF (different combinations) Coartem	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	Adults and children with uncomplicated Plasmodium falciparum malaria	Malaria patients 12 to < 18 years old with malaria caused by P. falciparum
Read-out Milestone(s)	H2-2021 (actual)	2023
Publication	CSR final in CREDI 20Dec2021	TBD

Abbreviations

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

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References

ENTRESTO	
1	IQVIA National Prescription Audit as of 25/03/2022.
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).
3	Zhang et al., ESC Heart Failure 2020; 7: 3841
4	Proudfoot et al., Int J Cardiol. 2021; 331:164
5	Including, but not limited to, the recent 2022 AHA/ACC/HFSA Guideline for the Management of Heart Failure (Heidenreich et al., J Am Coll Cardiol. 2022)

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1	Hauser S, et. al. Long-term safety of ofatumumab in patients with relapsing multiple sclerosis. Presented at American Academy of Neurology 2022 (S14.004).
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3	Ziemssen T, et al. KYRIOS clinical trial: Tracking the immune response to SARS-CoV-2 mRNA vaccines in an open-label multicenter study in participants with relapsing multiple sclerosis treated with ofatumumab s.c. Presented at American Academy of Neurology 2022, Levit E, et al. Mult Scler Relat Disord. 2022 Mar 3;60:103719
4	Based on SHP APLD representing 9K patients through Jan 2022
*	*IQVIA National Prescription Audit (NPA) data projected and scaled to IQVIA National Sales Perspectives (NSP) volume (national derivative of IQVIA Drug Distribution Data (DDD))

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