Financial Reporting

idorsia

The following information contains certain “forward-looking statements”, relating to the company’s business, which can be identified by the use of forward-looking terminology such as “estimates”, “believes”, “expects”, “may”, “are expected to”, “will”, “will continue”, “should”, “would be”, “seeks”, “pending” or “anticipates” or similar expressions, or by discussions of strategy, plans or intentions. Such statements include descriptions of the company’s investment and research and development programs and anticipated expenditures in connection therewith, descriptions of new products expected to be introduced by the company and anticipated customer demand for such products and products in the company’s existing portfolio. Such statements reflect the current views of the company with respect to future events and are subject to certain risks, uncertainties and assumptions. Many factors could cause the actual results, performance or achievements of the company to be materially different from any future results, performances or achievements that may be expressed or implied by such forward-looking statements. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those described herein as anticipated, believed, estimated or expected.

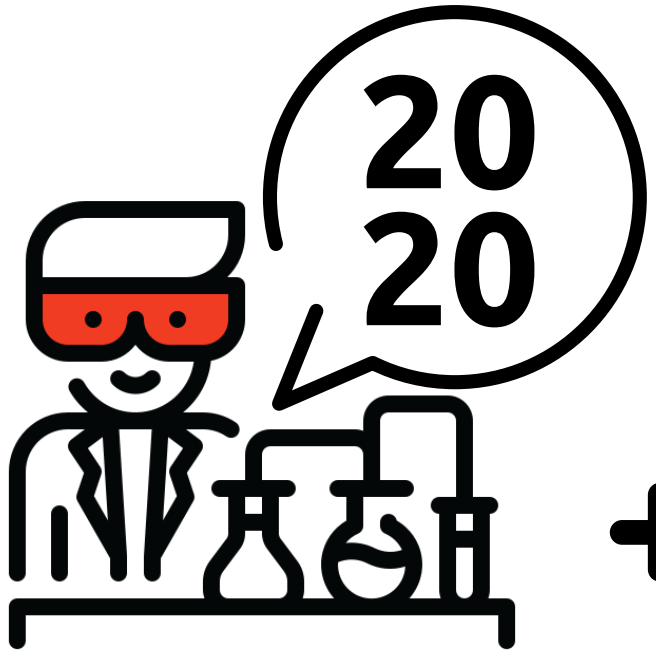
“I am very proud of all that has been accomplished at Idorsia in 2020.”

Jean-Paul Clozel
Chief Executive Officer

Highlights

Positive pivotal results with daridorexant for insomnia

Daridorexant NDA submitted to US FDA



US commercial operations established

Positive results in the Japanese registration program for clazosentan

Liquidity strengthened by equity raise of CHF 865 million

A rich development pipeline

Compound	Mechanism of Action	Target Indication	
Daridorexant	Dual orexin receptor antagonist	Insomnia	NDA submitted, MAA in preparation
Aprocitentan*	Dual endothelin receptor antagonist	Resistant hypertension management	Phase 3
Clazosentan	Endothelin receptor antagonist	Vasospasm associated with aneurysmal subarachnoid hemorrhage	Phase 3
Lucerastat	Glucosylceramide synthase inhibitor	Fabry disease	Phase 3
Selatogrel	$P2Y_{12}$ receptor antagonist	Suspected acute myocardial infarction	Phase 3 in preparation
Cenerimod	$S1P_1$ receptor modulator	Systemic lupus erythematosus	Phase 2
ACT-539313	Selective orexin 1 receptor antagonist	Binge eating disorder	Phase 2
Sinbaglustat	GBA2/GCS inhibitor	Rare lysosomal storage disorders	Phase 1 complete
ACT-1004-1239	CXCR7 antagonist	Immunology	Phase 1
ACT-1014-6470	-	Immunology	Phase 1
ACT-541478	-	CNS	Phase 1
ACT-777991	-	Immunology	Phase 1

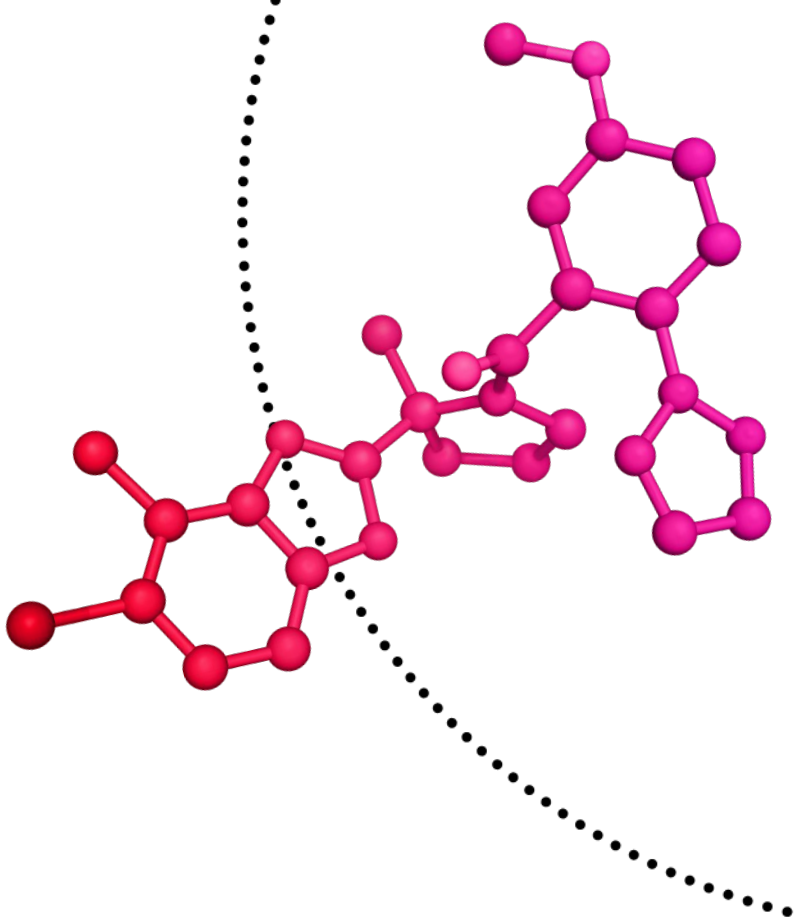
* In collaboration with Janssen Biotech to jointly develop aprocitentan, Janssen Biotech has sole commercialization rights worldwide.

Neurocrine Biosciences has a global license to develop and commercialize our ACT-709478, a novel T-type calcium channel blocker, for the treatment of a rare form of pediatric epilepsy. In November 2020, Neurocrine announced it had initiated a Phase 2 study for ACT-709478.

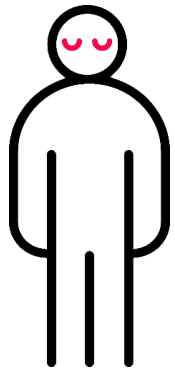
Daridorexant for insomnia

Dual orexin receptor antagonist

First non-sedative sleeping pill to demonstrate an improvement in sleep and daytime functioning as measured with a validated instrument, without compromising safety



- New drug application (NDA) submitted to US FDA January 2021
- Market authorization application (MAA) to be submitted to EU EMA in the coming months
- Commercial launch targeted for Q2 2022 as we begin the transformation and modernization of the insomnia market

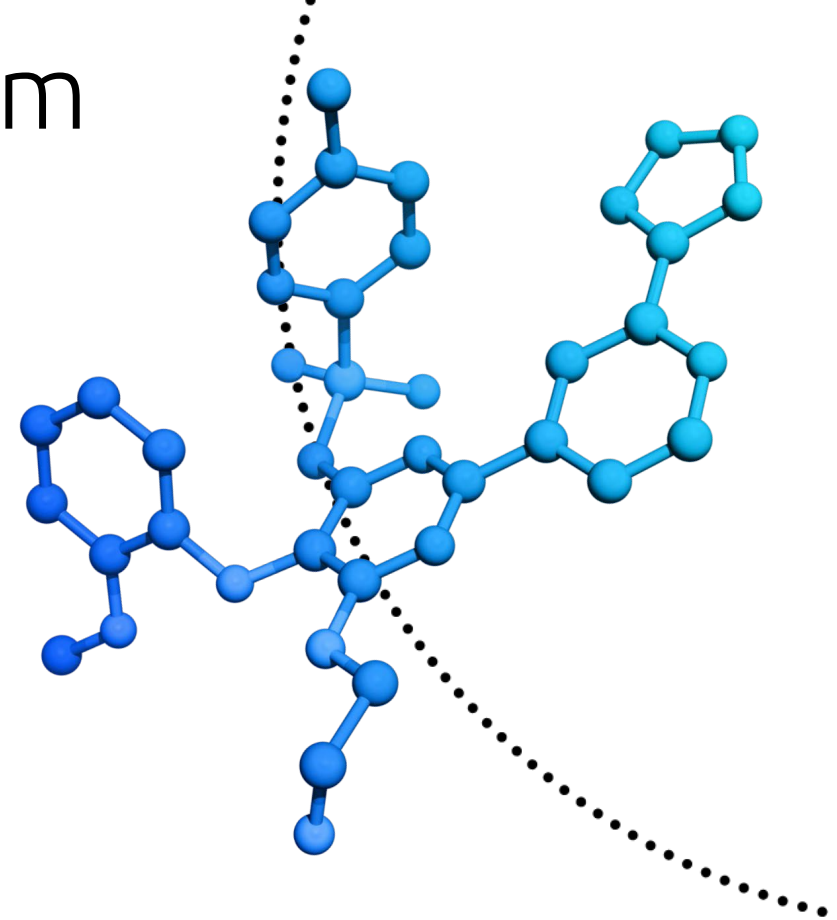


Daridorexant is investigational, in development and not approved or marketed in any country.

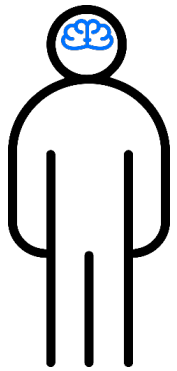
Clazosentan for cerebral vasospasm

Selective endothelin receptor (ET_A) antagonist

Novel pharmacological intervention to protect against cerebral ischemia post-subarachnoid hemorrhage



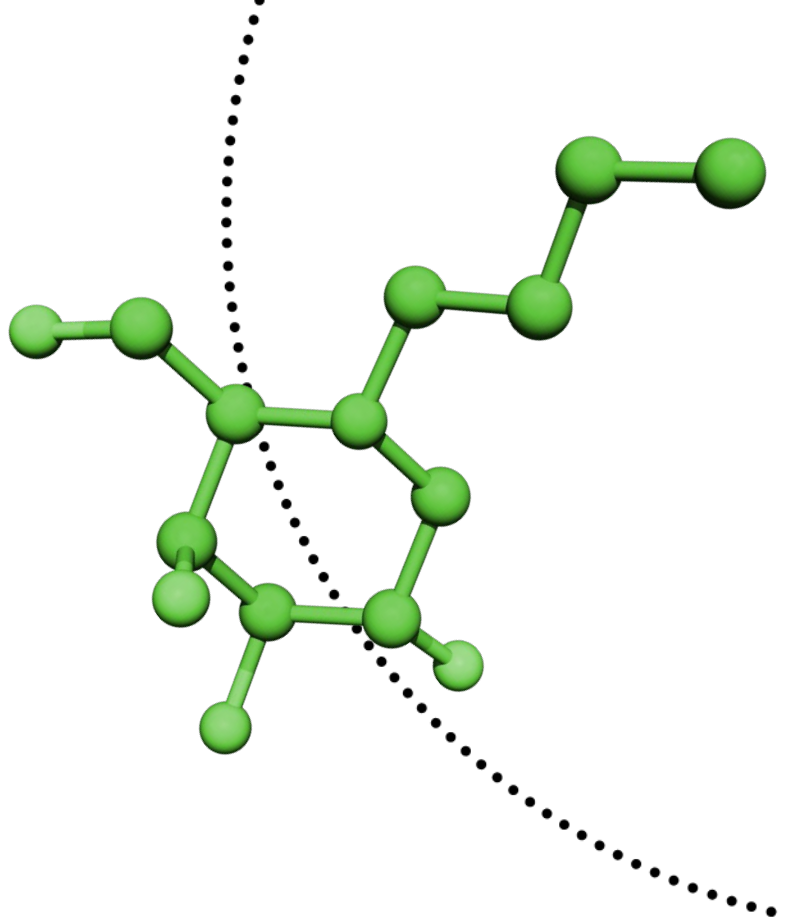
- Positive results in the Japanese registration program
- NDA to be filed with the PMDA in H1 2021
- REACT: Recruitment EU/US at the halfway point



Clazosentan is investigational, in development and not approved or marketed in any country.

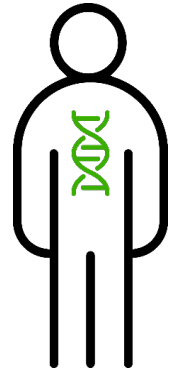
Lucerastat for Fabry disease

Glucosylceramide synthase inhibitor



Novel oral small molecule that can access tissues inaccessible to enzyme replacement therapy to reduce the damaging build-up of lipids irrespective of Fabry mutation

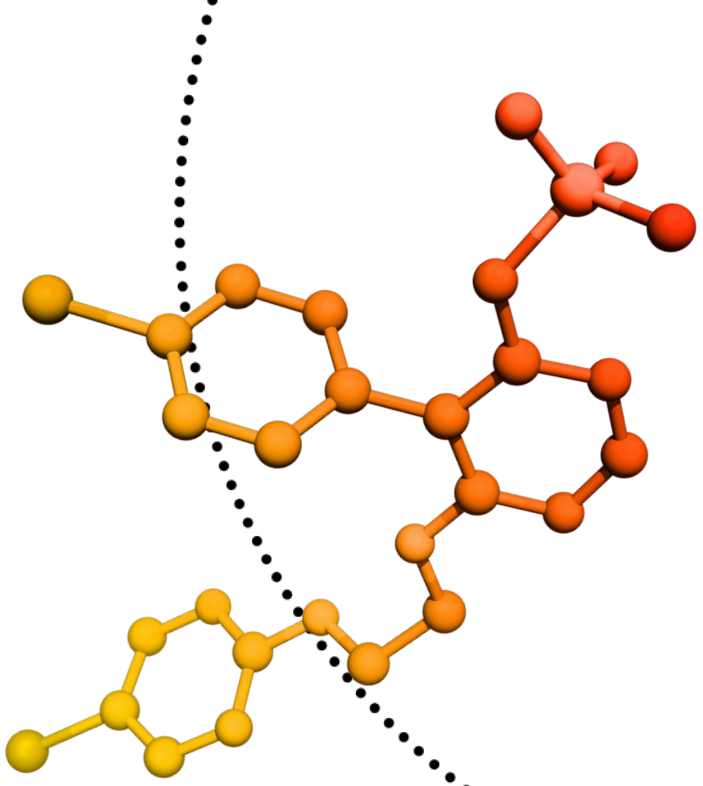
- MODIFY: recruitment complete
- Results expected in H2 2021



Lucerastat is investigational, in development and not approved or marketed in any country.

Aprocitentan for difficult-to-control hypertension

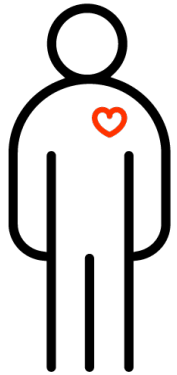
Dual endothelin receptor antagonist



Targeting the endothelin pathway for the first time in systemic hypertension has great potential for the treatment of difficult-to-control or “resistant” hypertension

- PRECISION: recruitment complete
- Results expected in H1 2022

janssen
PHARMACEUTICAL COMPANIES OF
Johnson & Johnson



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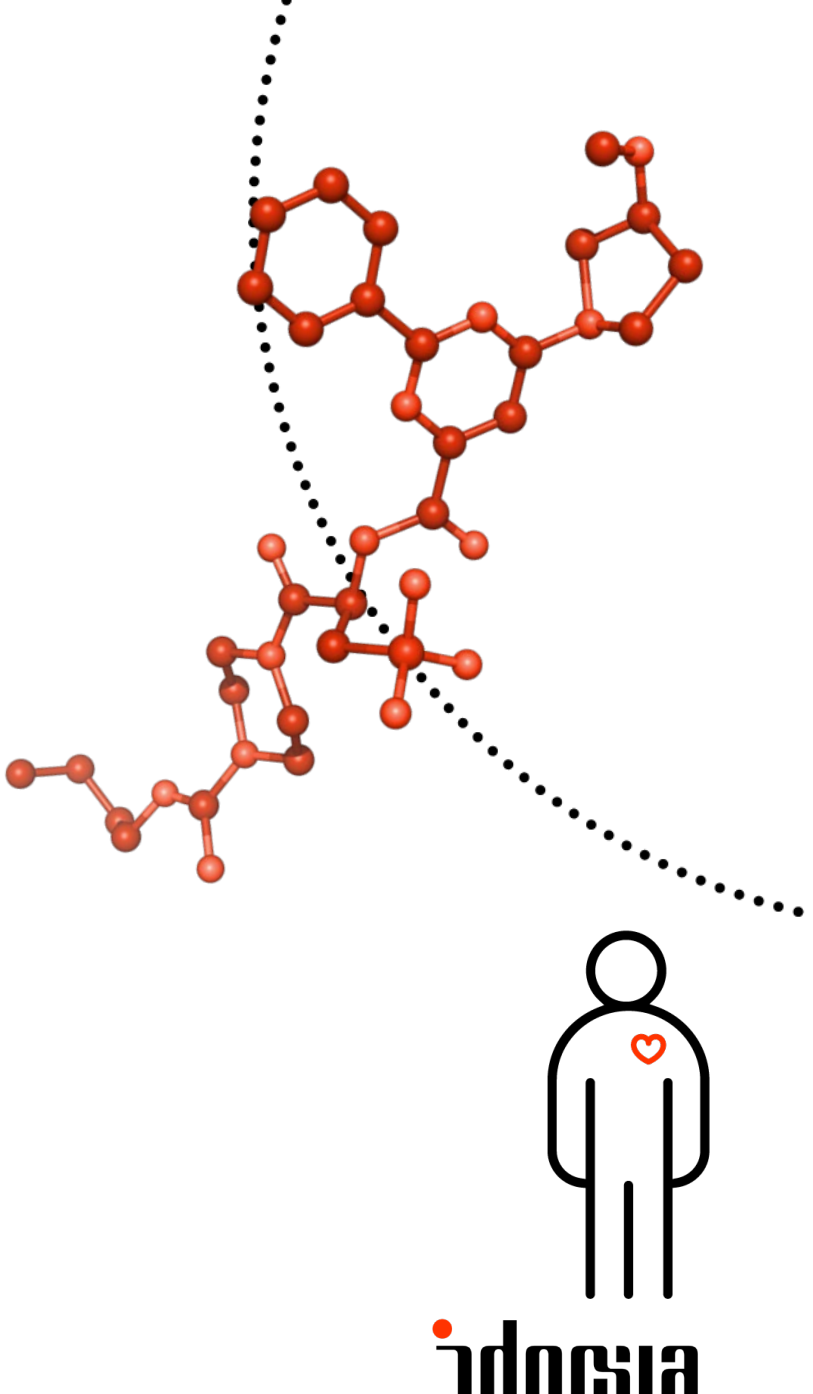
Aprocitentan is investigational, in development and not approved or marketed in any country.

Selatogrel for suspected AMI

Highly-selective $P2Y_{12}$ receptor antagonist

Novel emergency, self-administered agent to protect heart muscle due to heart attack in the crucial time between symptom onset and first medical attention, so as to prevent severe clinical outcomes



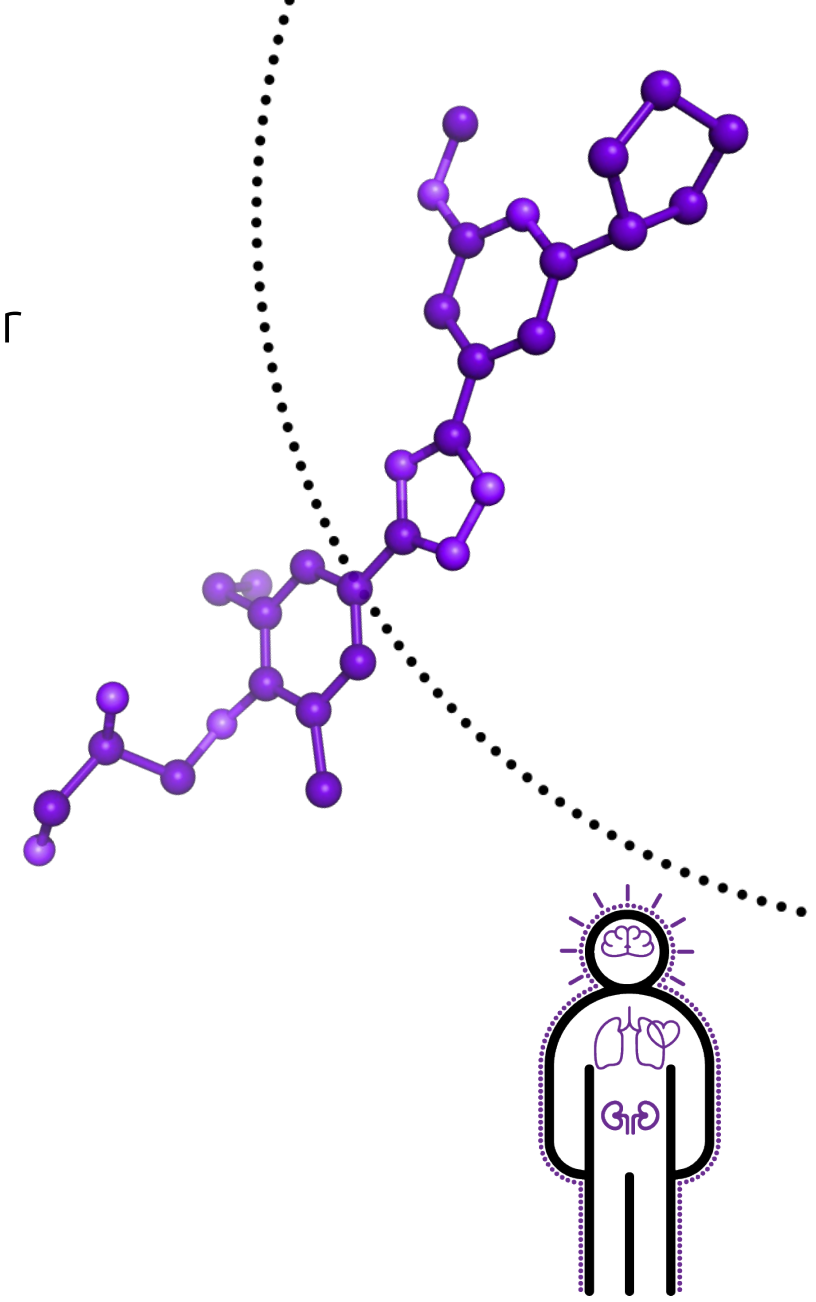
- Special Protocol Assessment (SPA) agreed with the FDA
- "Fast-track" designation received from FDA
- Phase 3 study "SOS-AMI" – target enrollment of approx. 14'000 patients – to be initiated mid-2021

Selatogrel is investigational, in development and not approved or marketed in any country.

Cenerimod for systemic lupus erythematosus

Selective sphingosine-1-phosphate 1 ($S1P_1$) receptor modulator

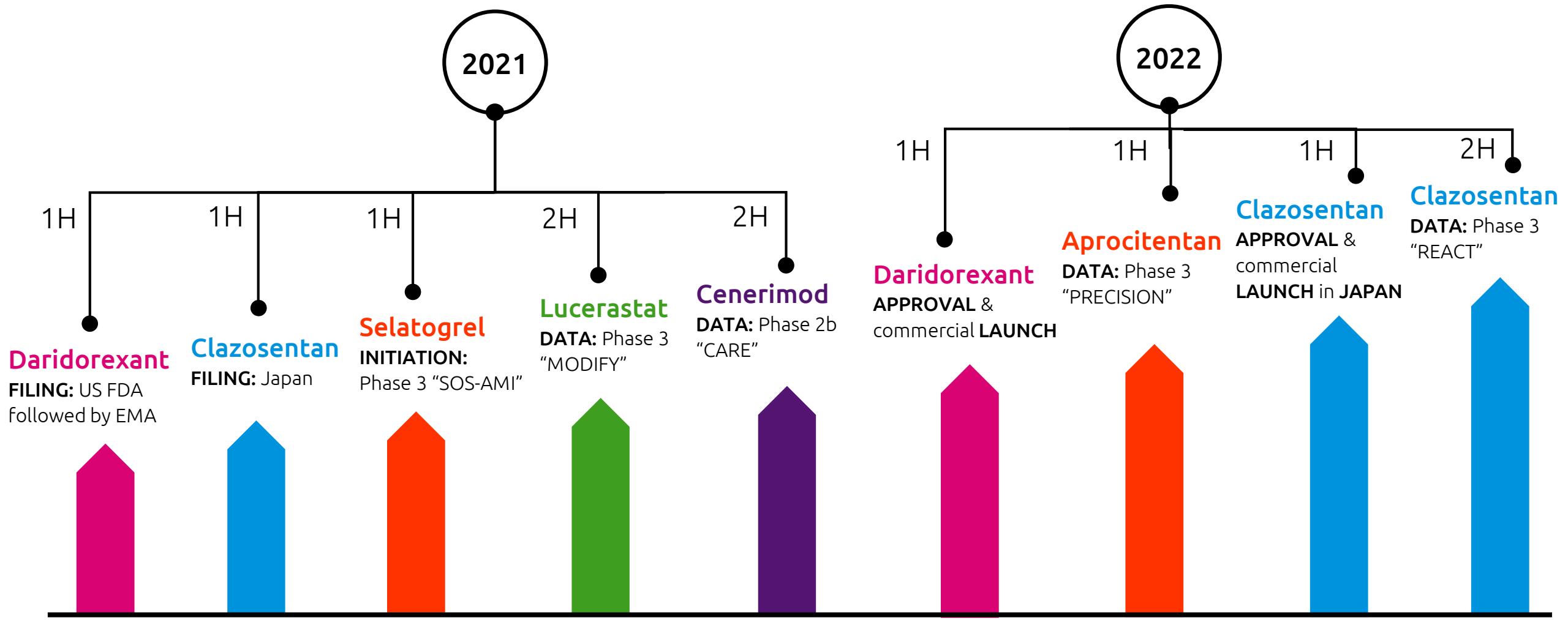
First oral immune-modulator active on both B and T cells could represent a novel approach to the treatment of SLE



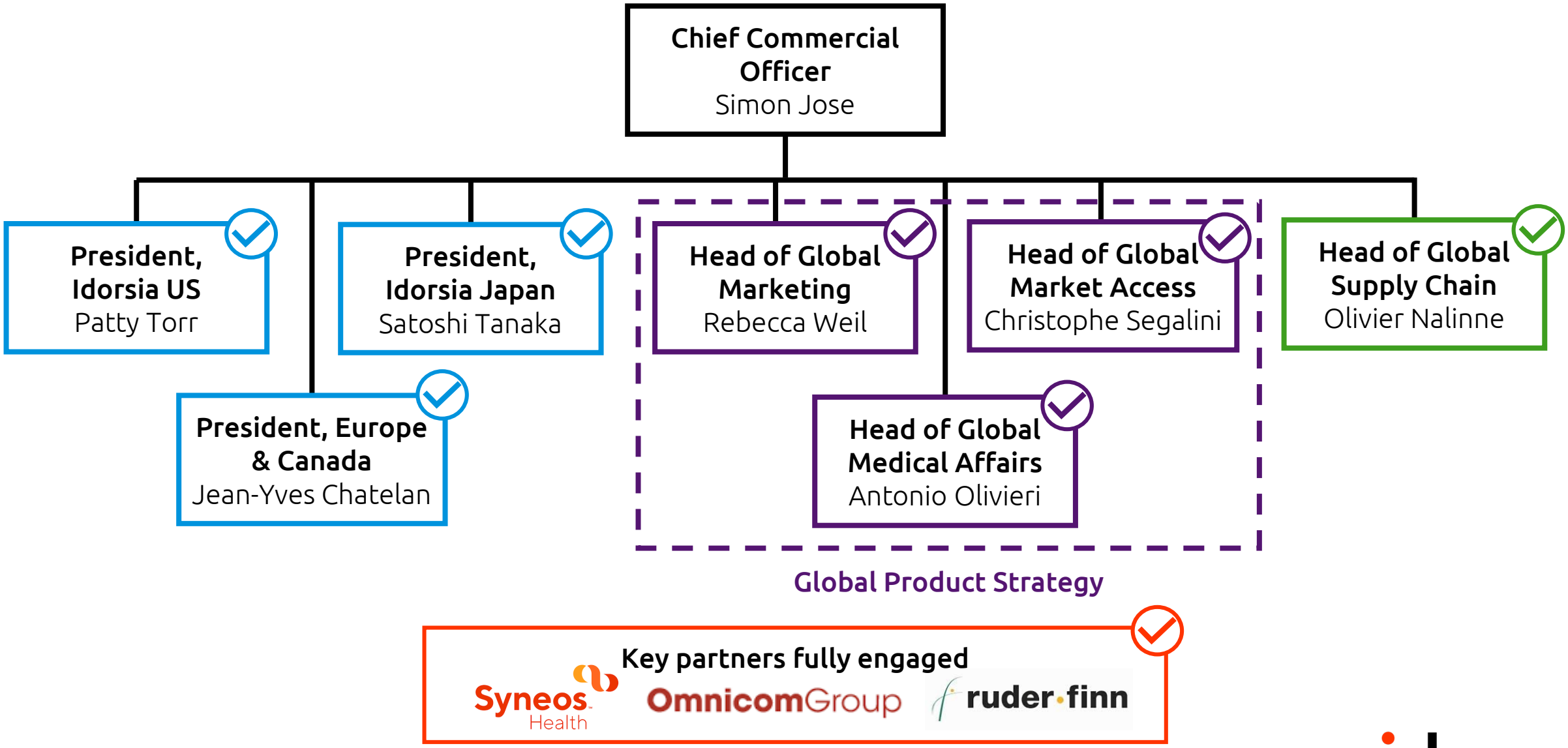
- "Fast-track" designation received from FDA
- CARE: recruitment complete by end-Feb
- Results expected in H2 2021

Cenerimod is investigational, in development and not approved or marketed in any country.

Idorsia is at an inflection point with major catalysts expected in the near-term



Building a commercial organization

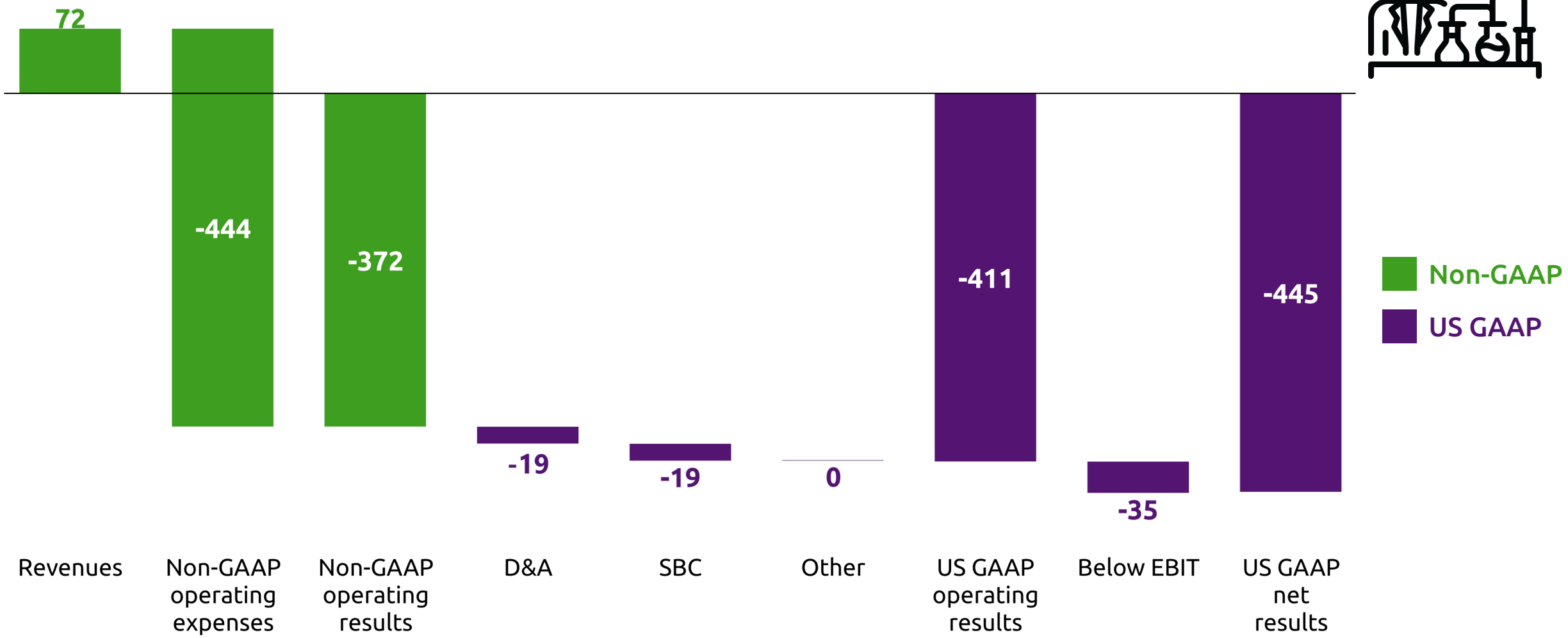


“Our strengthened balance sheet with CHF 1.2 billion liquidity will take us through to the next inflection points.”

André C. Muller
Chief Financial Officer

US GAAP net results

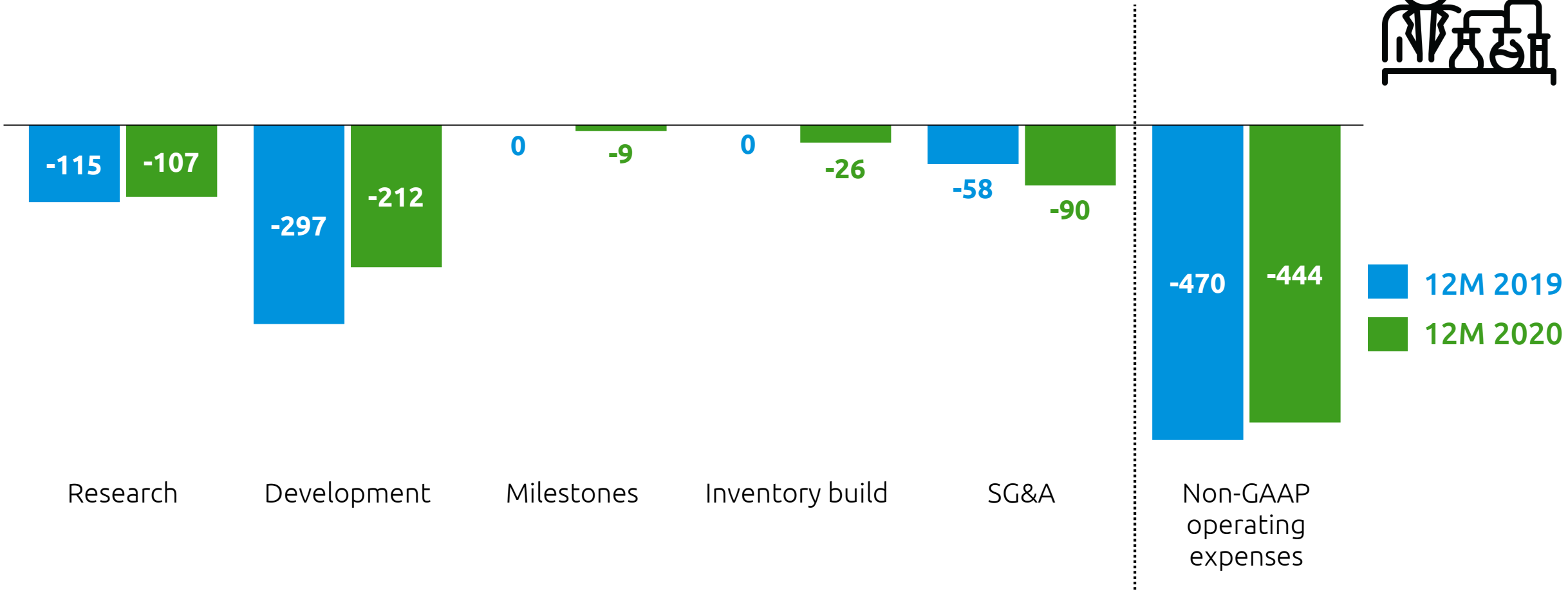
in CHF millions, rounding differences may occur



Financial results as of December 31, 2020

Non-GAAP operating expenses

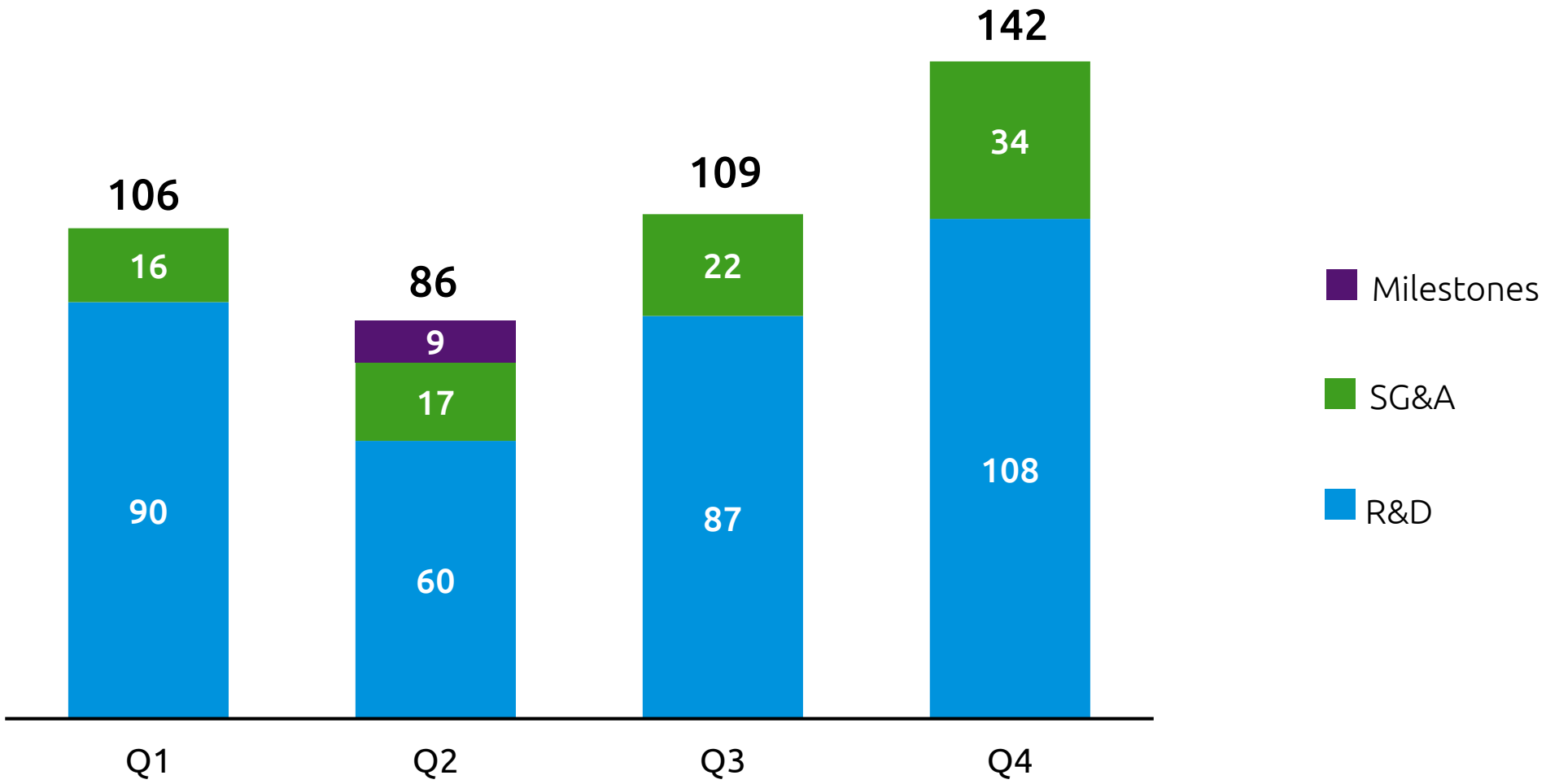
in CHF millions, rounding differences may occur



Financial results as of December 31, 2020

Quarterly Non-GAAP operating expenses

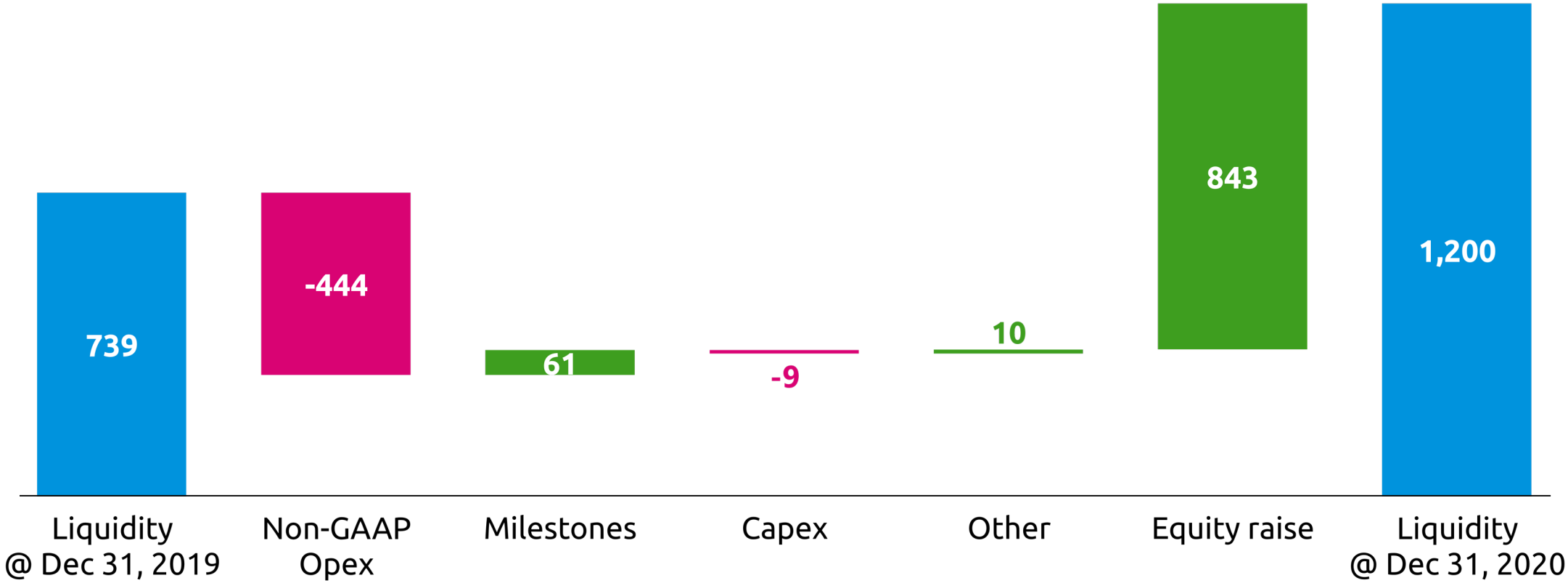
in CHF millions, rounding differences may occur



Financial results as of December 31, 2020

Cash flow

in CHF millions, rounding differences may occur



Financial results as of December 31, 2020

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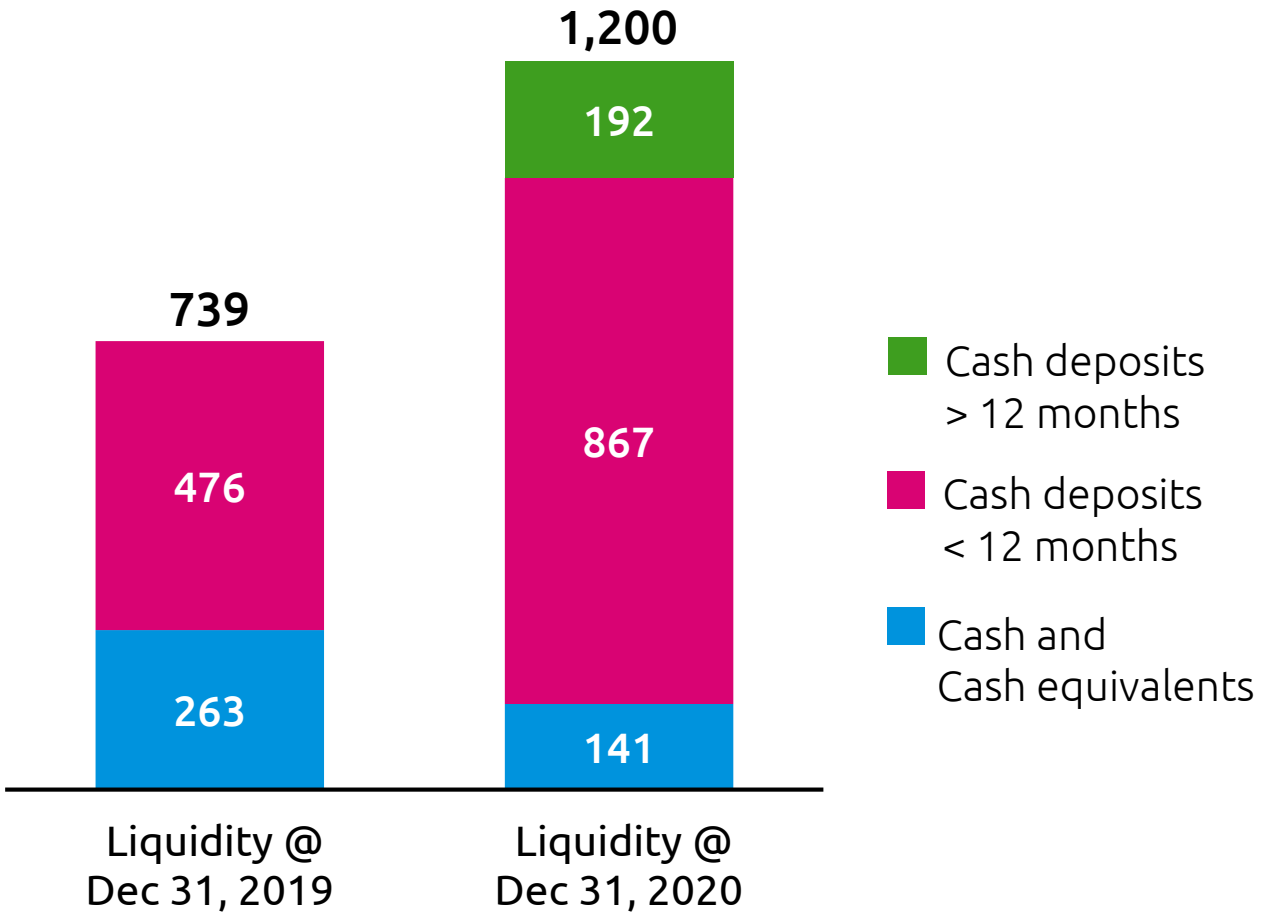
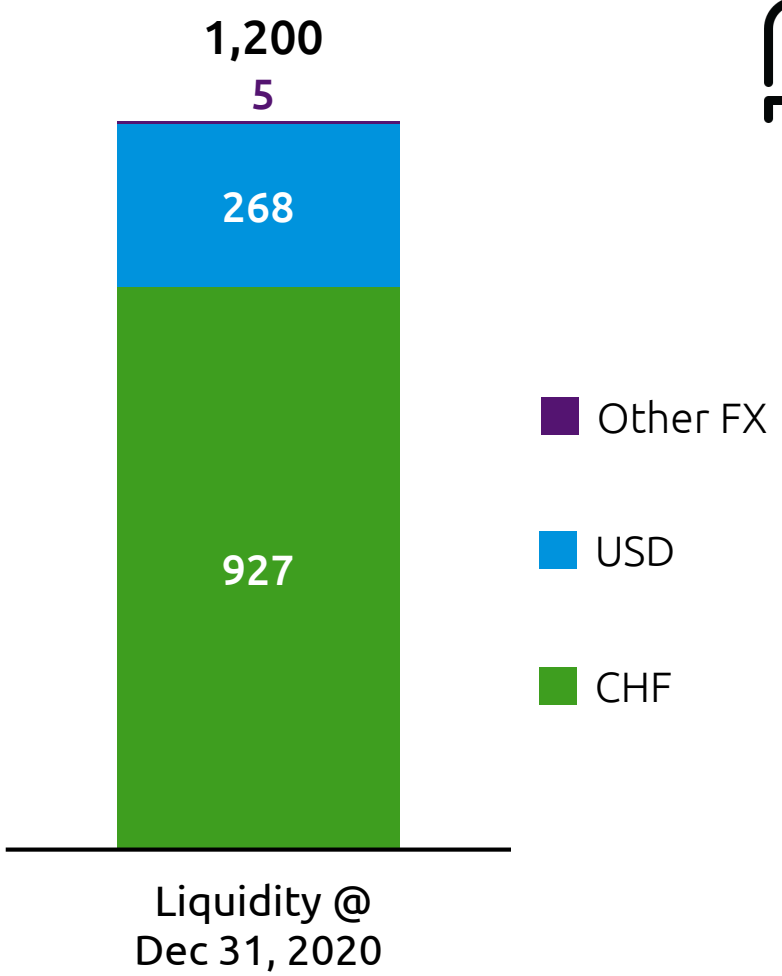
Liquidity

in CHF millions, rounding differences may occur





Financial results as of December 31, 2020

Financial Guidance for 2021

- Functional R&D expenses
  ~ CHF 370 million
- Functional SG&A
  ~ CHF 230 million
- Inventory build
  ~ CHF 35 million
- Milestone payment
  CHF 5 million

- Non-GAAP OPEX
- Depreciation & Amortization
  ~ CHF 20 million
- Stock-Based Compensation
  ~ CHF 25 million

Non-GAAP operating expenses*
~ **CHF 640 million**

US GAAP operating expenses*
~ **CHF 685 million**

* Excluding unforeseen events

Idorsia's potential first revenue stream

Ponesimod for relapsing multiple sclerosis

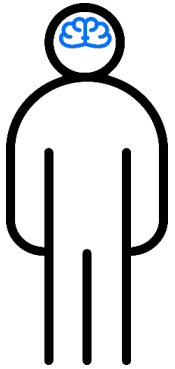
Phase 3 data presented by Janssen

Ponesimod showed **superiority versus Aubagio** (teriflunomide) 14 mg in adults with relapsing multiple sclerosis

Janssen submitted an NDA and MAA for ponesimod in March 2020

Revenue sharing agreement

- Idorsia is entitled to receive **8% of revenues** of ponesimod if marketed



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Ponesimod is investigational, in development and not approved or marketed in any country.

Idorsia revenues in the future

Rich pipeline allows substantial leverage of the commercial organization

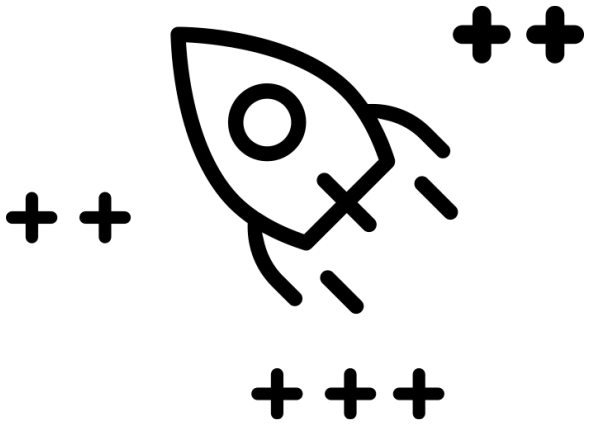
Net sales

- **GP Product:** daridorexant
- **Orphan:** lucerastat, clazosentan
- **Specialty:** cenerimod, selatogrel

Milestones & Royalty streams

- ponesimod
- aprocitentan
- T-type calcium channel blocker

2021 is a key year for Idorsia



2021

- Enter 2021 with a **strong balance sheet**
- Filing of **daridorexant** in the US and EU
- PDUFA of **ponesimod** for J&J
  Idorsia has a revenue-sharing agreement in respect to ponesimod
- Filing of **clazosentan** in Japan
- Start Phase 3 (under SPA) for **selatogrel**
- Phase 3 results for **lucerastat** in Fabry disease
- Phase 2b results for **cenerimod** in SLE

“2021 is key for our vision to build a sustainable mid-sized pharmaceutical company.”

Jean-Paul Clozel
Chief Executive Officer