Idorsia – Reaching out for more

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.

“We have one simple vision: creating a sustainable mid-size pharma company based on innovation”

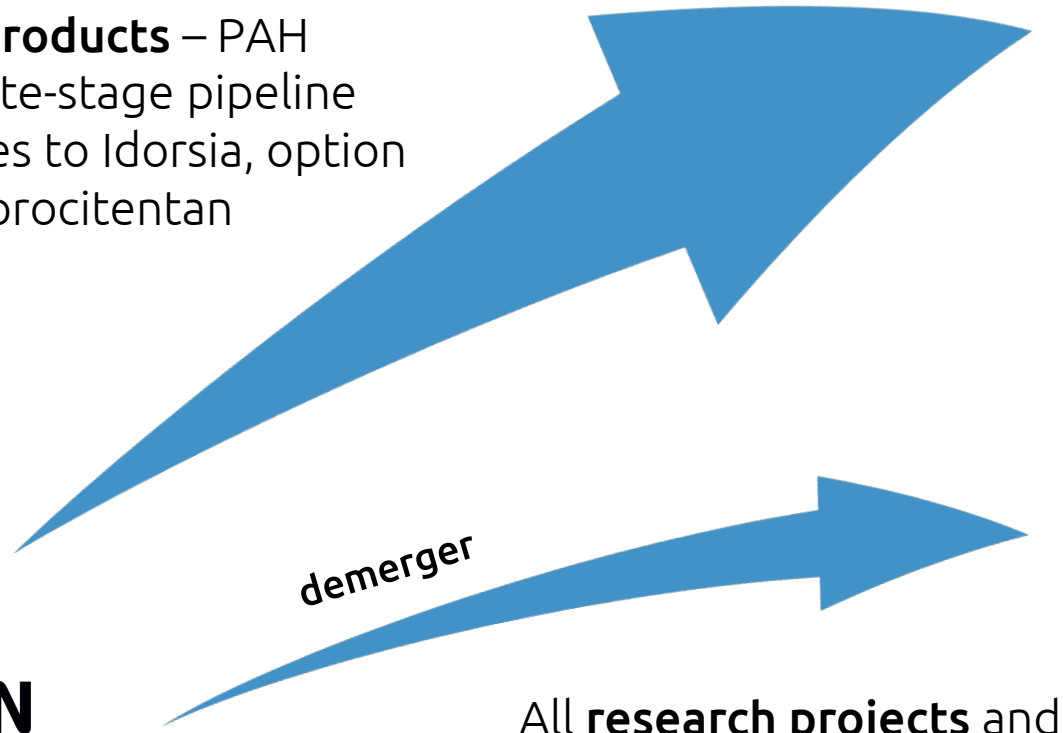
Jean-Paul Clozel
Chief Executive Officer

The origin of Idorsia

USD 30 Billion acquisition of Actelion – Idorsia is born

Marketed products – PAH franchise, late-stage pipeline with royalties to Idorsia, option to license aprocitentan

Johnson & Johnson
Family of Companies



ACTELION

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All **research projects** and **early-stage pipeline**

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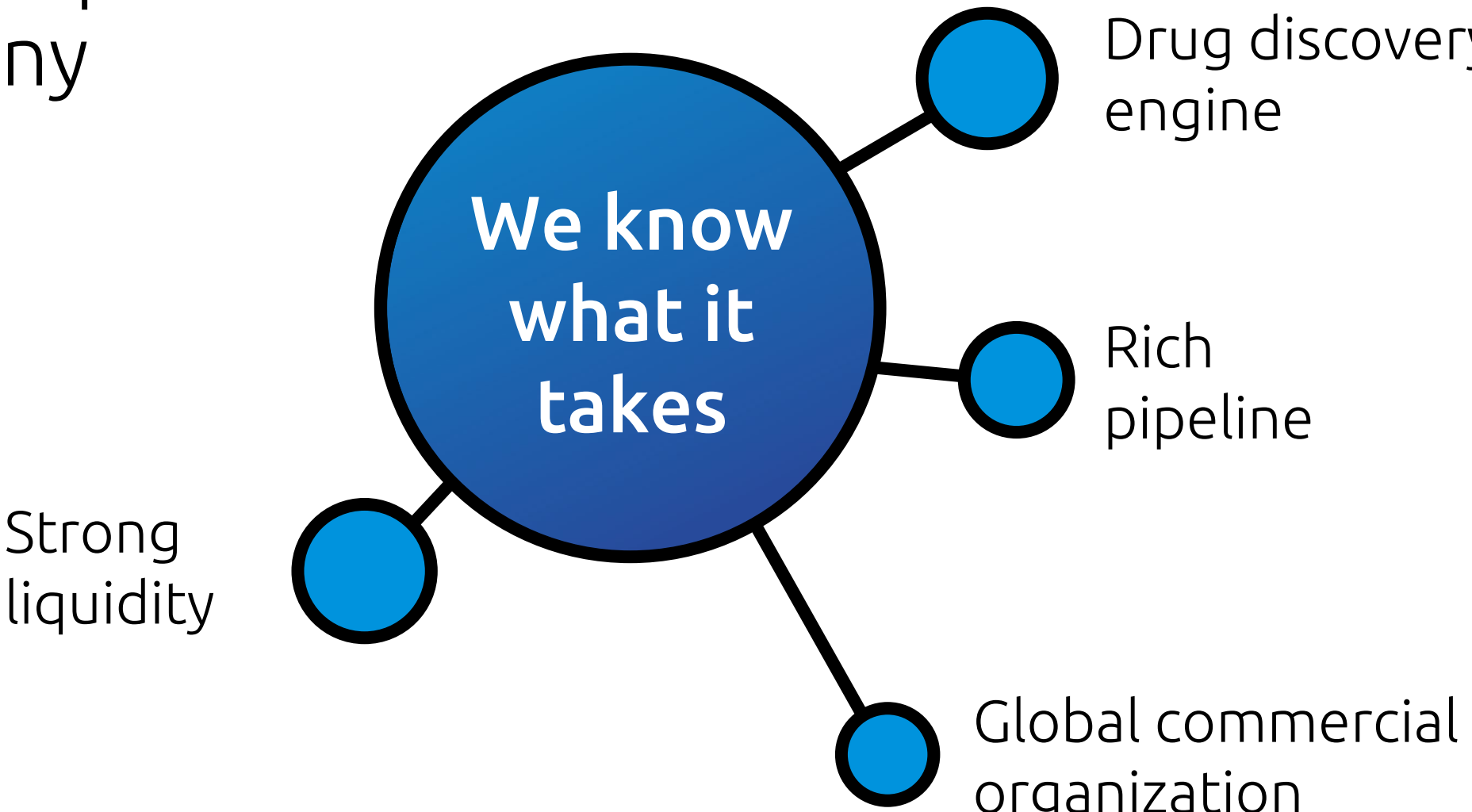
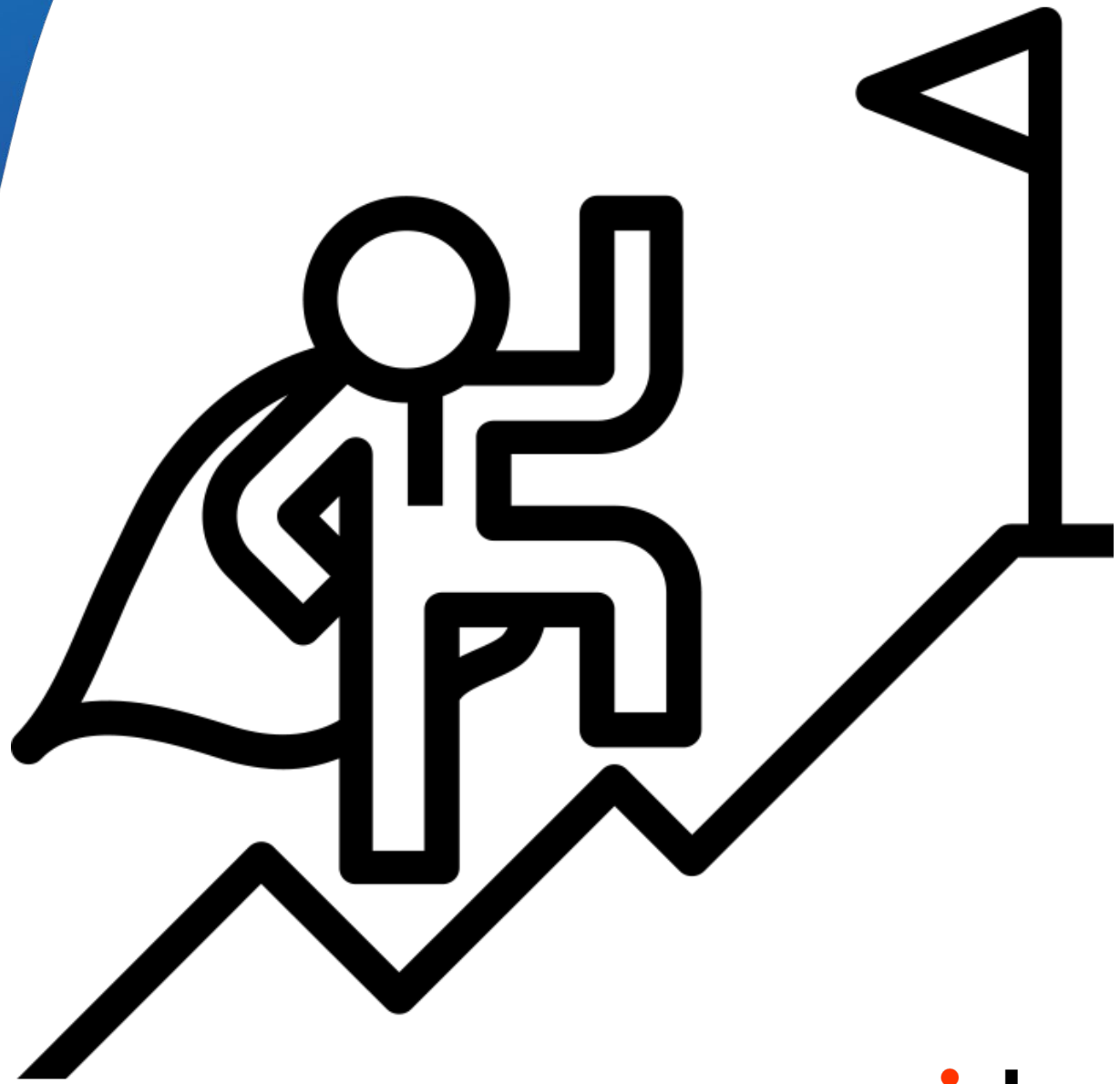
Idorsia today: A 3-year-old biotech with a 20-year heritage

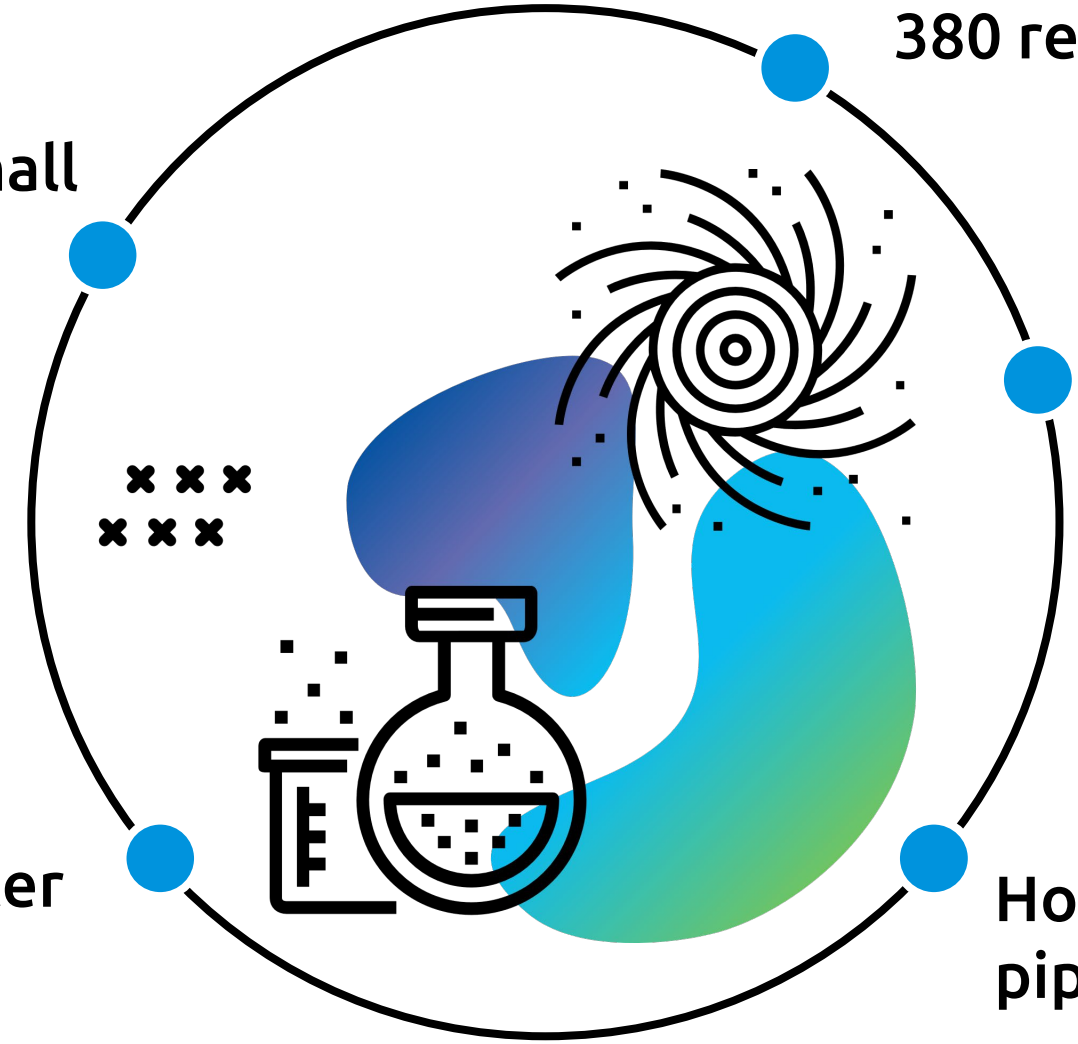
Becoming a sustainable mid-size pharma company



Despite COVID-19 Idorsia made progress on all fronts in 2020



Our drug discovery engine



380 researchers

Discovering
2 – 3 New Chemical
Entities (NCE) / year

Home-grown
pipeline

Single-center
approach

Focus on small
molecules

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-774312	CRTH2 receptor antagonist	Nasal polyposis	Phase 2
ACT-539313	Selective orexin 1 receptor antagonist	Psychiatric disorders	Phase 2 in preparation
Sinbaglustat	GBA2/GCS inhibitor	Rare lysosomal storage disorders	Phase 1 complete
ACT-1004-1239	CXCR7 antagonist	Immunology / Cancer immunotherapy	Phase 1
ACT-1014-6470	-	Immunology	Phase 1
ACT-541478	-	CNS	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.

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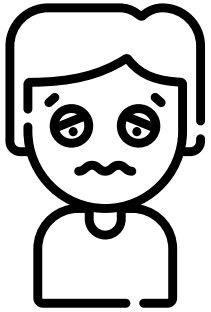
Daridorexant

Insomnia market

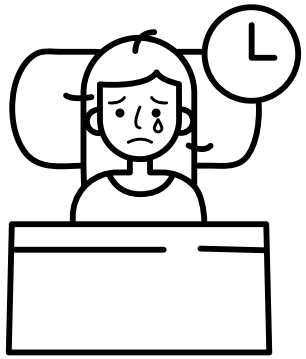
Large opportunity in a **highly unsatisfied market**

~10%
of adults suffer from insomnia

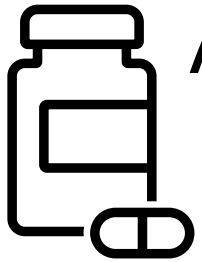


16 million
Treated patients in the US alone

1.5 billion USD
is 5% of the insomnia market at suvorexant list price



200+
billion USD
cost of insomnia to the US economy



Almost all generic

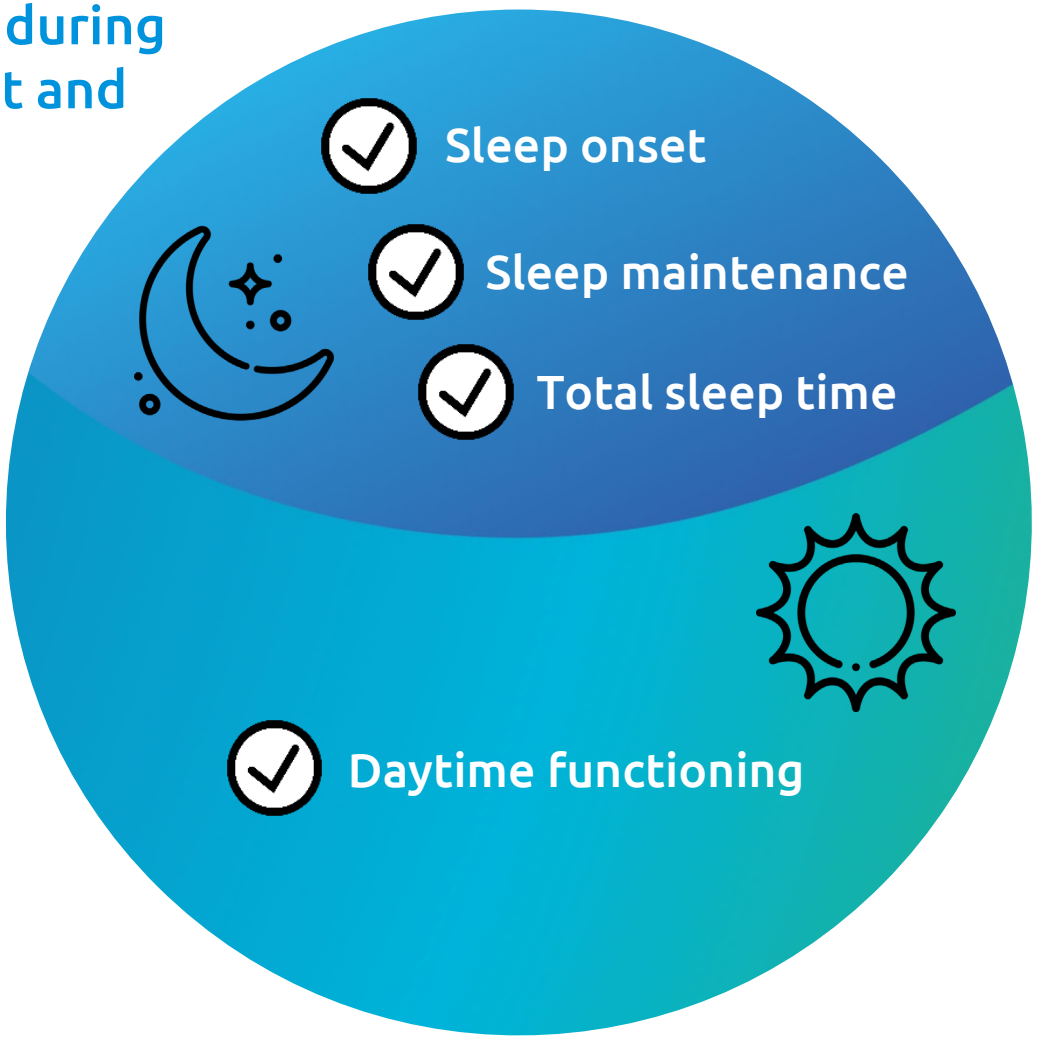
- Decline in z-drug use since 2013 FDA warnings
- Corresponding increase in off-label trazodone use

At least
14 years
of patent life for daridorexant

Daridorexant – Phase 3 registration program

Revolutionizing the treatment of insomnia

Efficacy during the night and the day



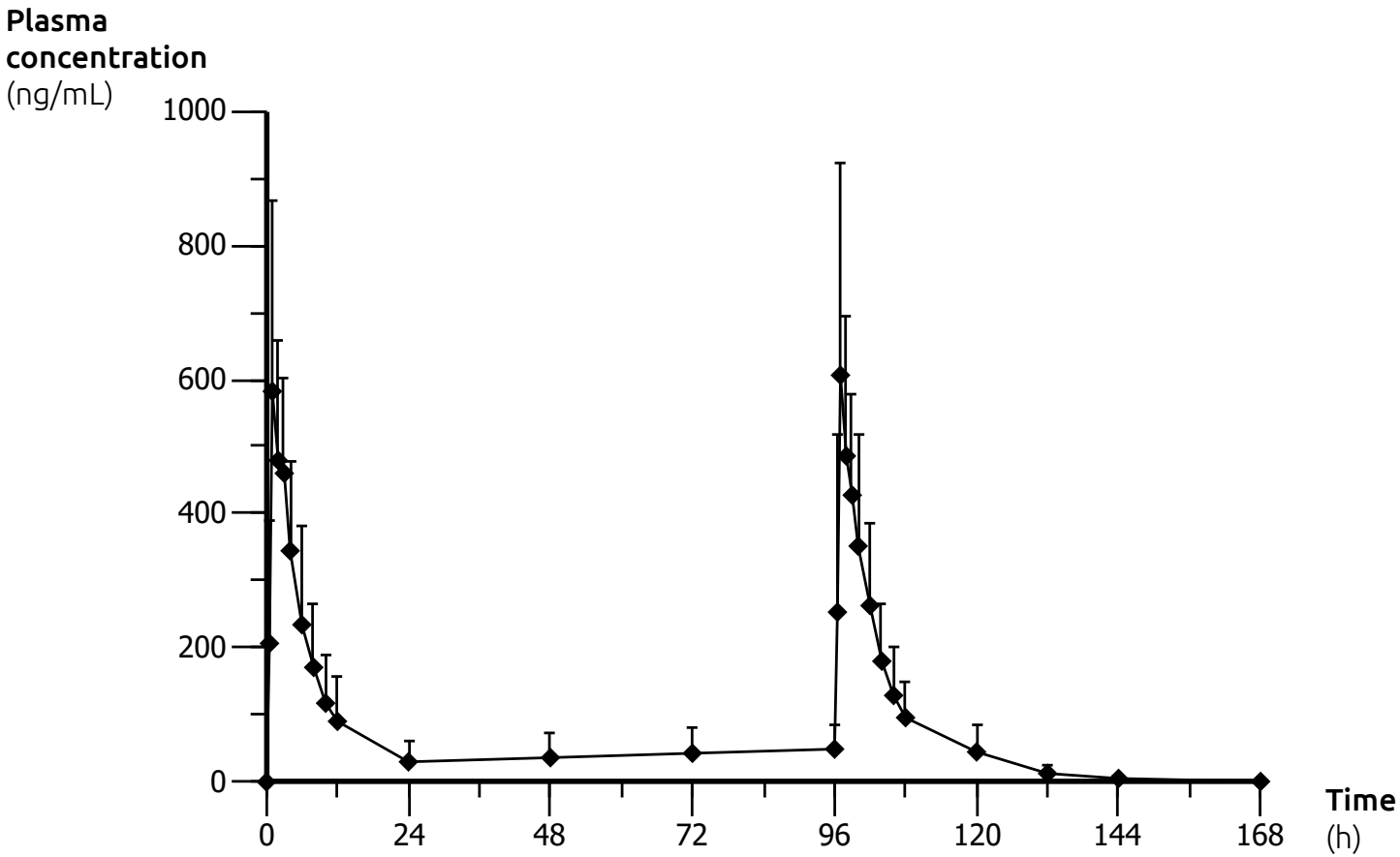
Safety and tolerability profile consistent between both pivotal studies

- No dose-dependent treatment emergent adverse events
- Low rate of clinically relevant adverse events
- No next morning hang-over effect
- No sign of rebound insomnia
- No withdrawal symptoms

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

Different by design – next generation DORA

Optimized pharmacokinetic profile

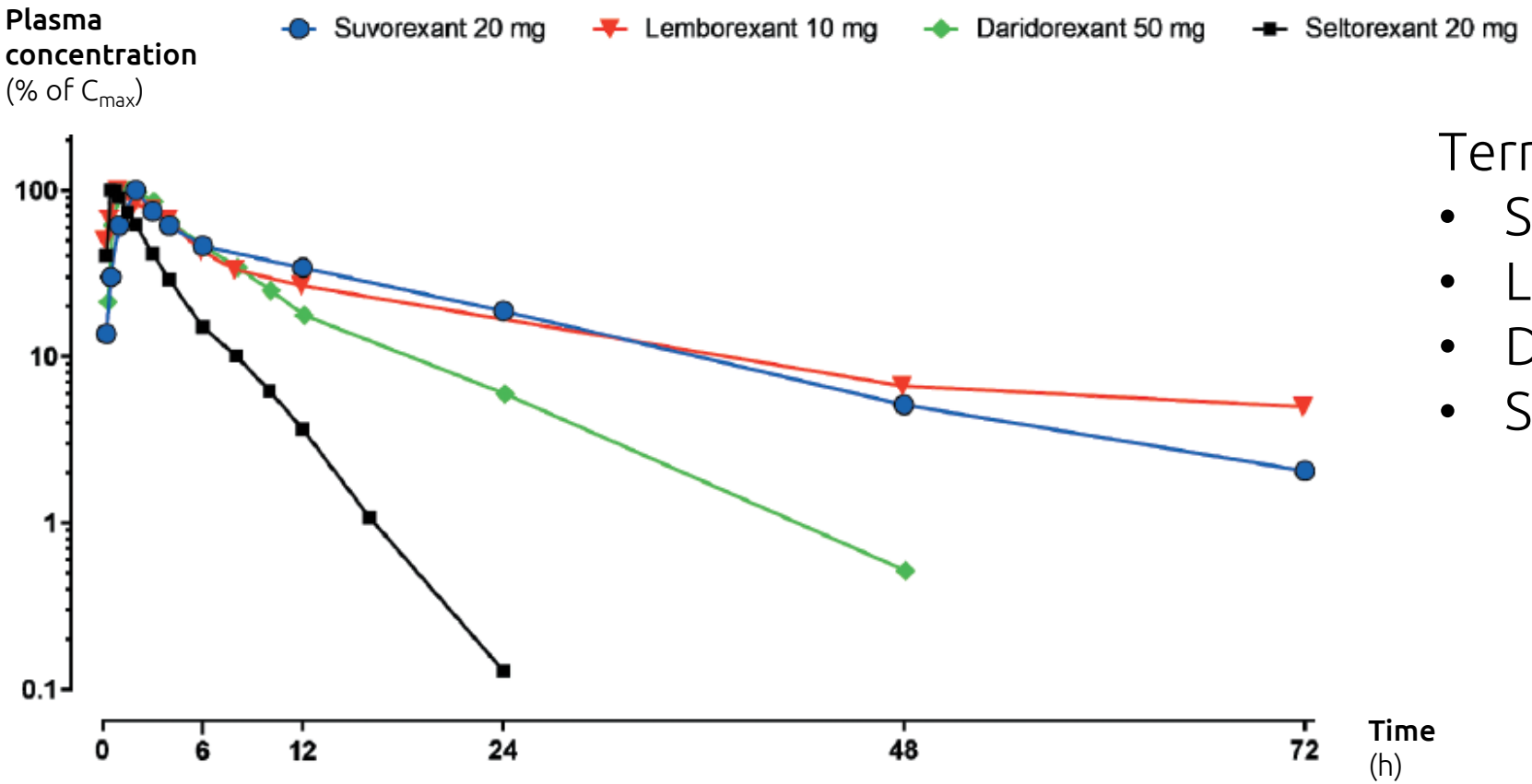


- Fast absorption
- Optimal half-life (8 h)
- No accumulation over time
- No active metabolites

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

Different by design – next generation DORA

C_{max} – normalized concentration-time profiles



Terminal elimination half-life:

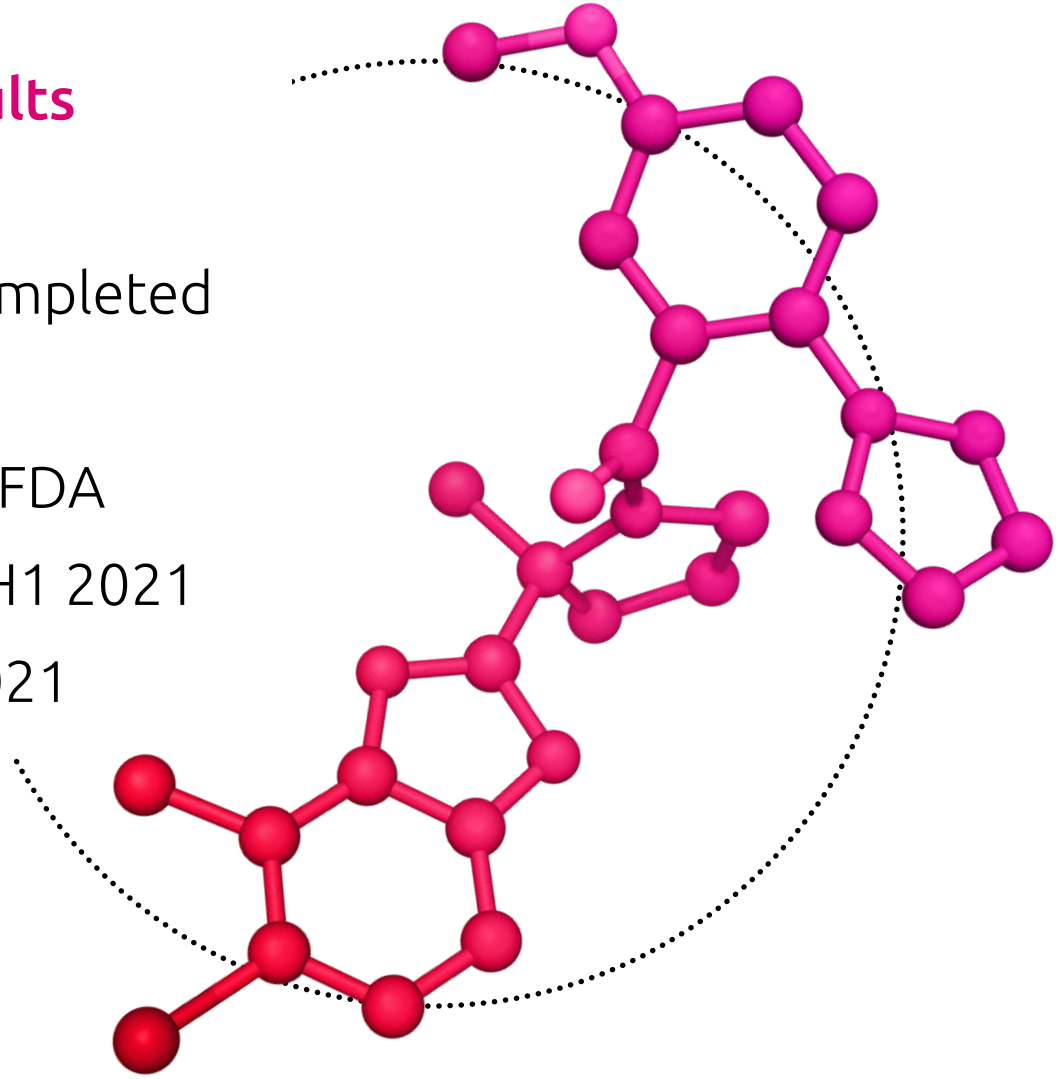
- Suvorexant 12 h
- Lemborexant 55 h
- Daridorexant 8 h
- Seltorexant 2.5 h

C_{max} = maximum plasma concentration.
The data on the basis of which these profiles were constructed are taken from scientific publication.

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

Advancing towards registration

- ✓ Two pivotal Phase 3 studies with **positive results**
- ✓ **Long-term efficacy and safety** confirmed
- ✓ Very **large clinical pharmacology program** completed
- ✓ **Phase 3 in Japan** initiated with Mochida
- ✓ New drug application **(NDA) submitted** to US FDA
- ✓ **Commercial pre-launch activity** will begin in H1 2021
- ✓ **European MAA submission** planned for H1 2021

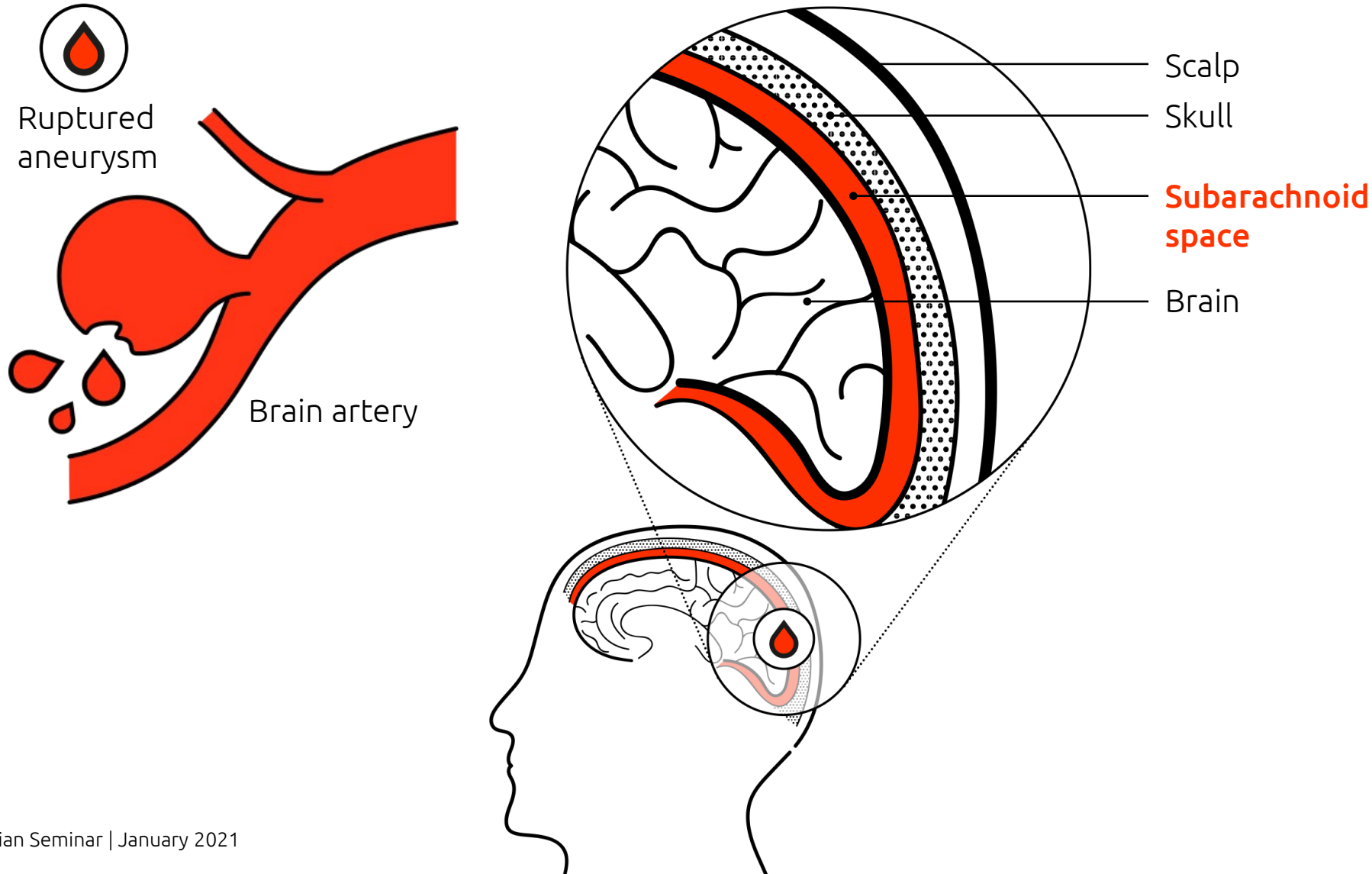


Daridorexant is investigational, in development and not approved or marketed in any country.

Clazosentan

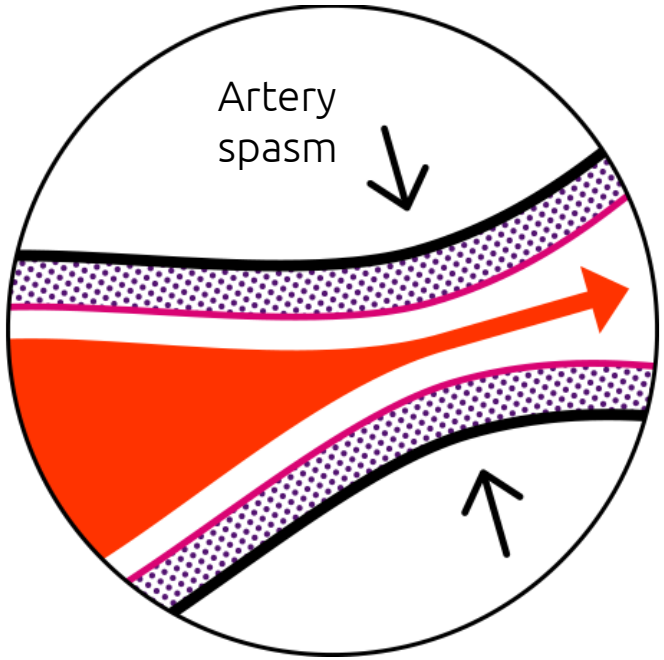
Aneurysmal subarachnoid hemorrhage (aSAH)

A sudden life-threatening bleeding occurring in the subarachnoid space



Cerebral vasospasm post-aSAH

Occurs between 4 and 14 days after aSAH securing

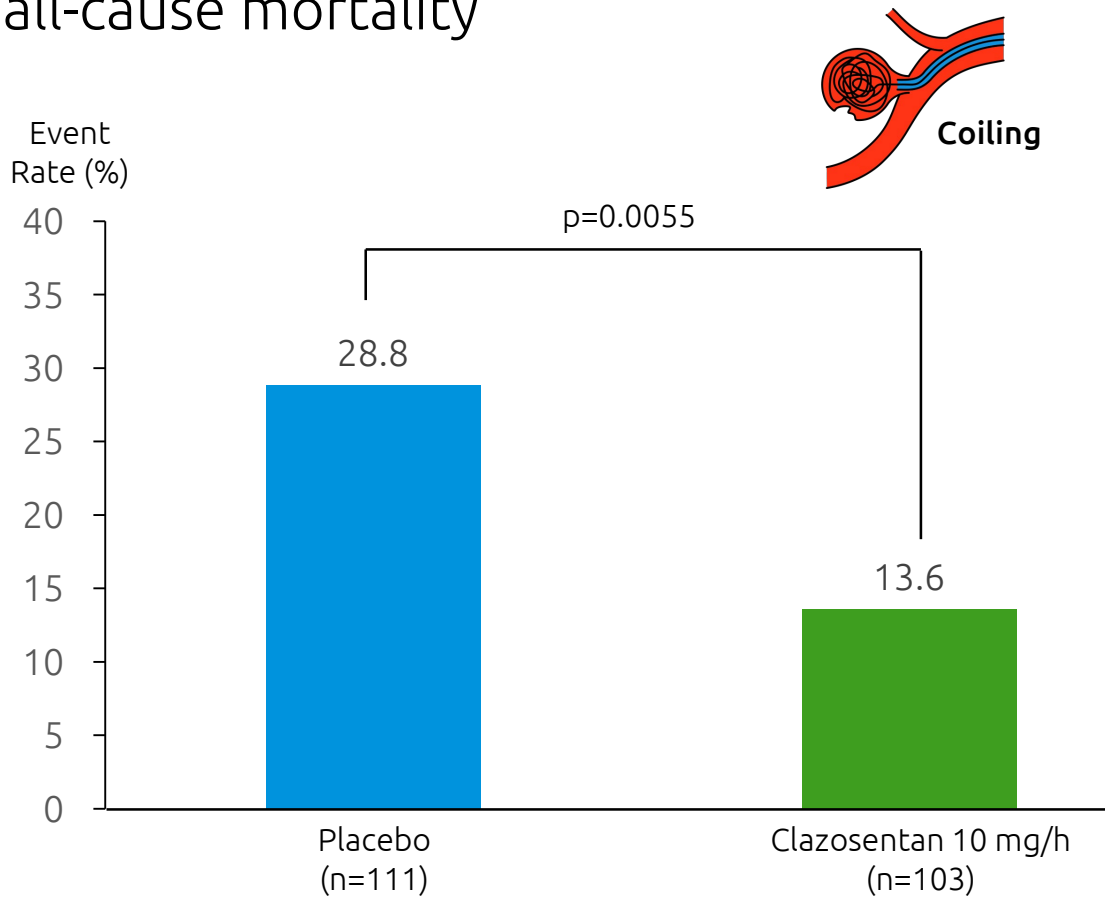


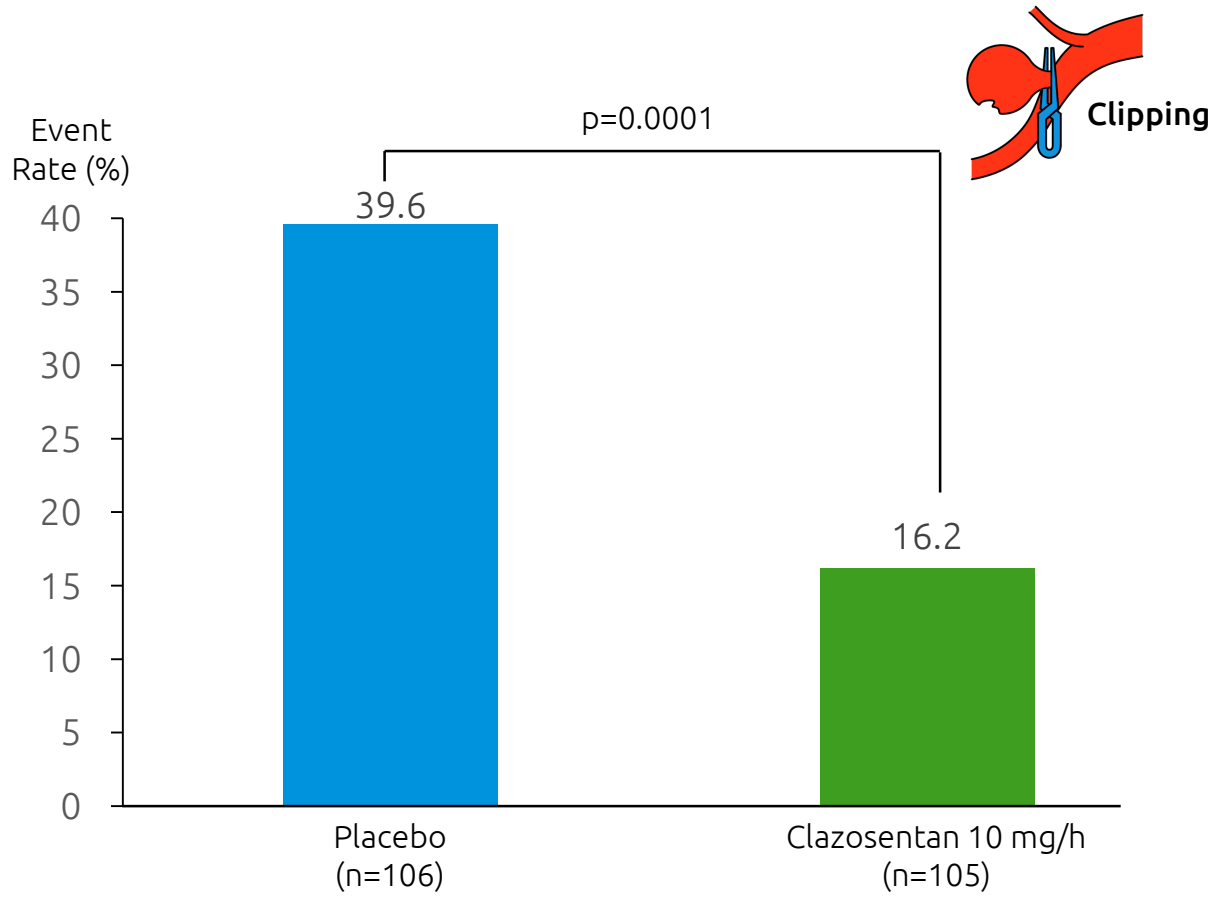
Baseline aSAH: normal MCA

7 day after SAH: cerebral vasospasm

Japanese clazosentan registration program

Significant effect on primary endpoint: Incidence of vasospasm-related morbidity and all-cause mortality





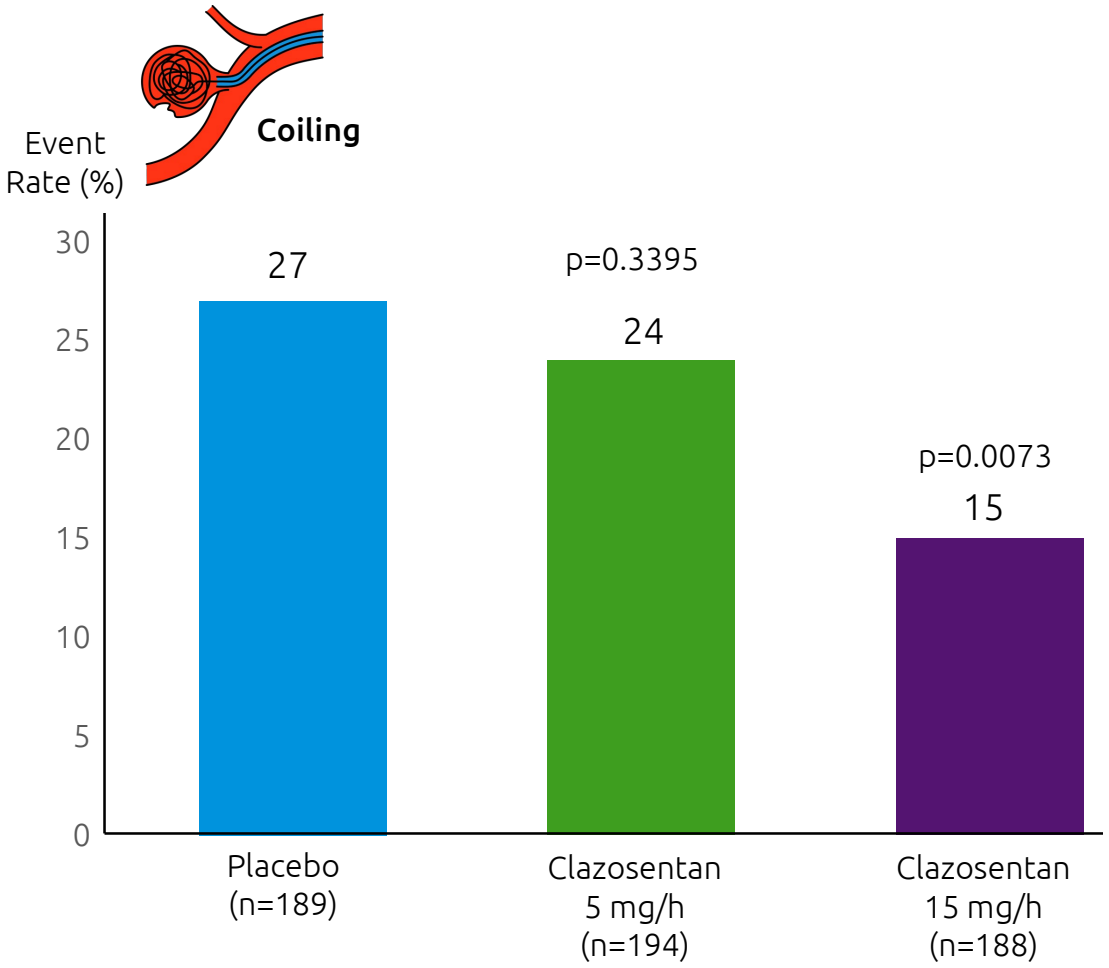
Full Analysis Set
Statistical test: Cochran-Mantel-Haenszel test stratified by WFNS Grade (pre-procedure)

No unexpected safety findings. TEAEs occurring >5% in the clazosentan group with a difference of >2% compared to placebo were vomiting and signs of hemodilution or fluid retention.

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

Clazosentan in CONSCIOUS-3 study

Exploratory analysis* 15mg/hr showed significant effect on morbidity / mortality



*Recruitment into CONSCIOUS 3 was concluded early in October 2010

Randomized Trial of Clazosentan in Patients With Aneurysmal Subarachnoid Hemorrhage Undergoing Endovascular Coiling

R. Loch Macdonald, MD, PhD; Randall T. Higashida, MD; Emanuela Keller, MD; Stephan A. Mayer, MD; Andy Molyneux, MD; Andreas Raabe, MD; Peter Vajkoczy, MD; Isabel Wanke, MD; Doris Bach, MSc; Aline Frey, PharmD; Pegah Nowbakht, PhD; Sébastien Roux, MD; Neal Kassell, MD

Stroke. 2012; 43(6):1463-9

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

Selatogrel

Selatogrel for subcutaneous self-administration

The “Cardiac Pen”

>800,000

heart attacks in the US every year

8.4

million survivors

According to the American Heart Association, there are currently 8.4 million heart attack survivors in the US

Special Protocol Assessment (SPA) has been agreed with the FDA

“fast-track” designation from FDA received

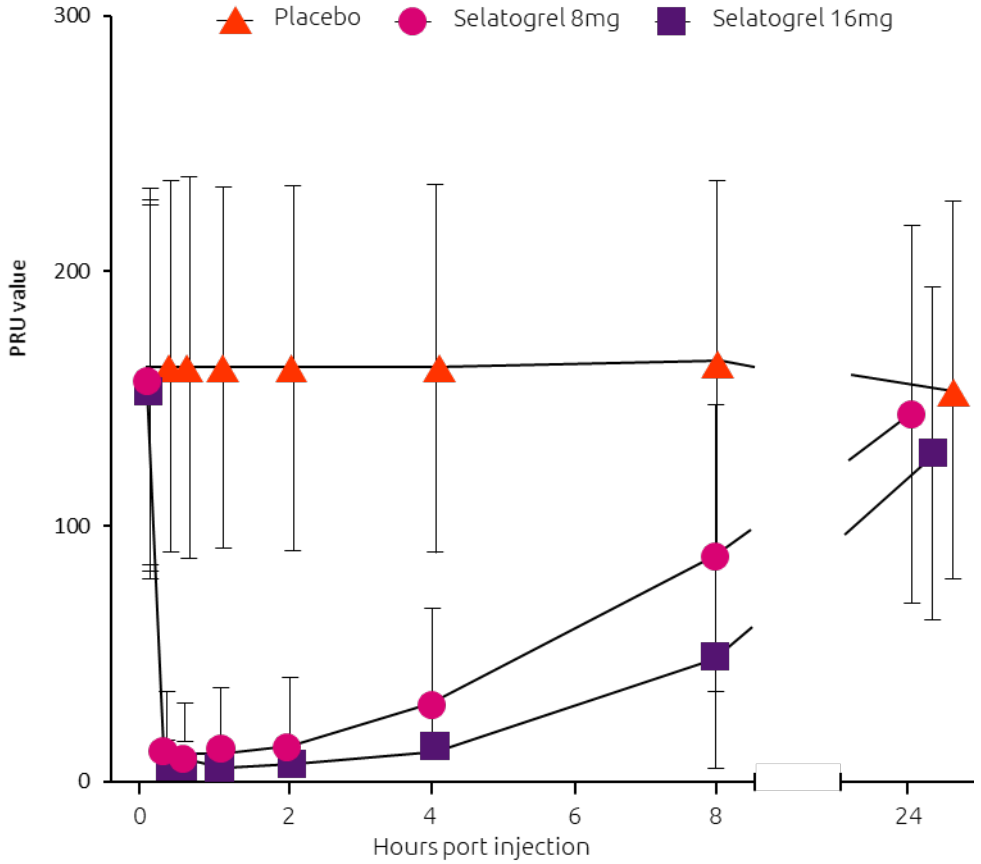
SOS-AMI: Phase 3 study with **14’000 patients** expected to be initiated in **H1 2021**

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

Phase 2 data with selatogrel

Selatogrel has a rapid PD effect following subcutaneous injection

- Subcutaneous administration of selatogrel 8 mg and 16 mg has demonstrated a rapid onset of action, **within 15 minutes**, with the height of its effect **extending over 4-8 hours**, depending on the dose
- Selatogrel was **safe and well tolerated** in both studies and there were no treatment-emergent serious bleeds

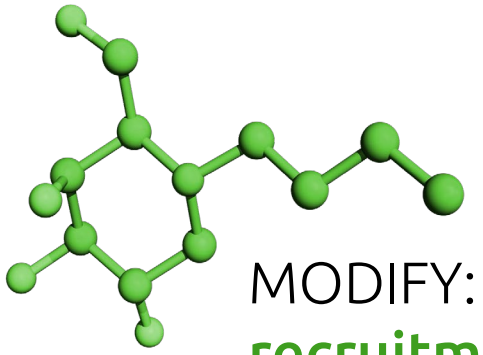


PRU: Platelet reactivity unit

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

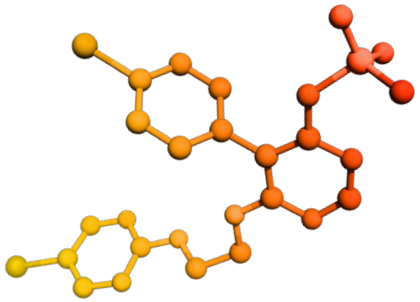
Additional major achievements in 2020

Late-stage pipeline advancing



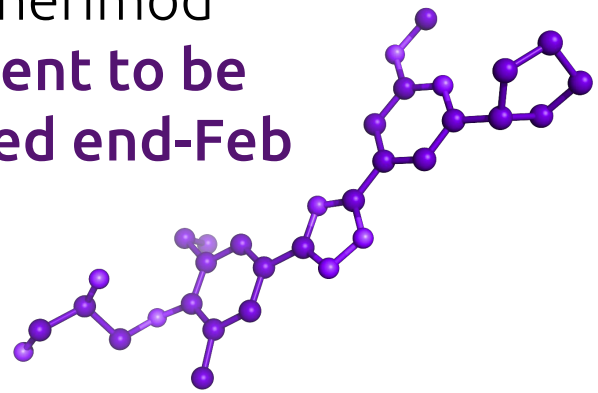
MODIFY: lucerastat
recruitment completed

REACT: clazosentan in EU/US
recruitment halfway completed



PRECISION: aprocitentan
recruitment nearing completion – within days!

CARE: cenerimod
recruitment to be completed end-Feb



All these projects faced multiple DSMB reviews with no drug-related safety signals flagged

Lucerastat, clazosentan, aprocitentan, and cenerimod are investigational, in development and not approved or marketed in any country.

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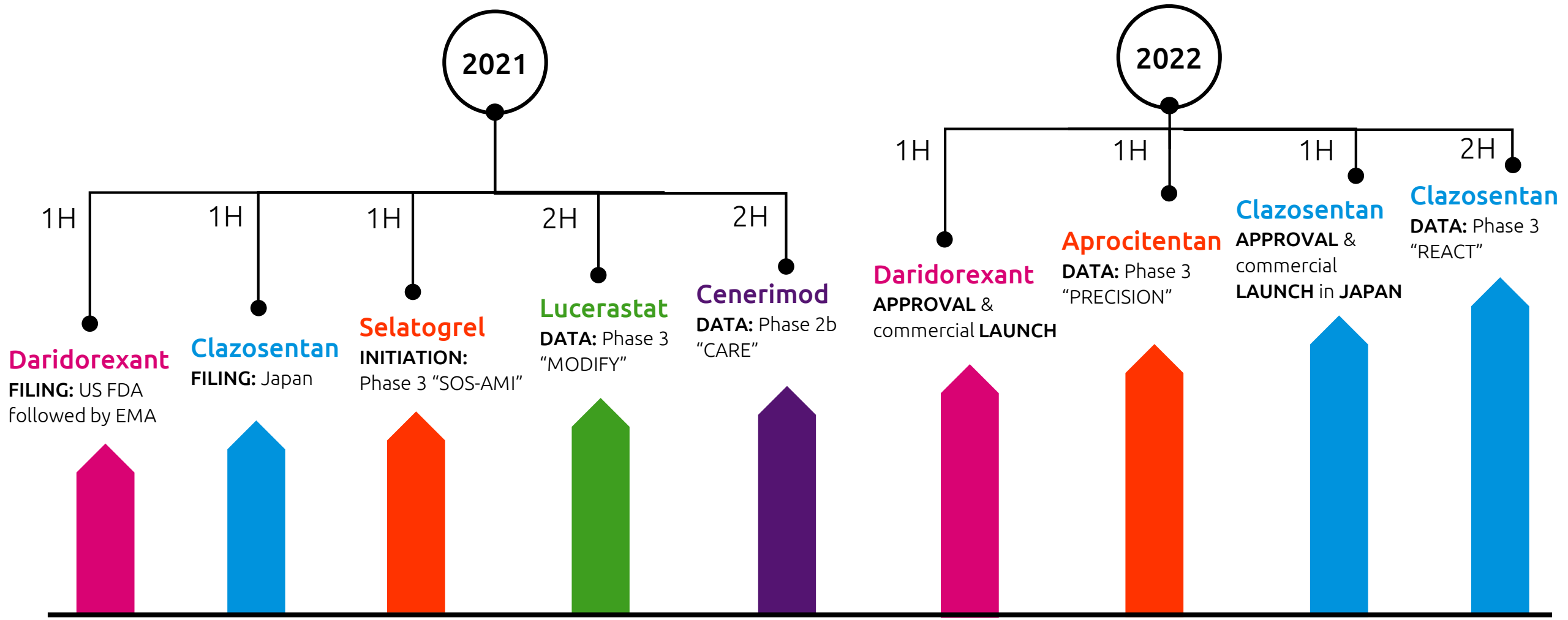
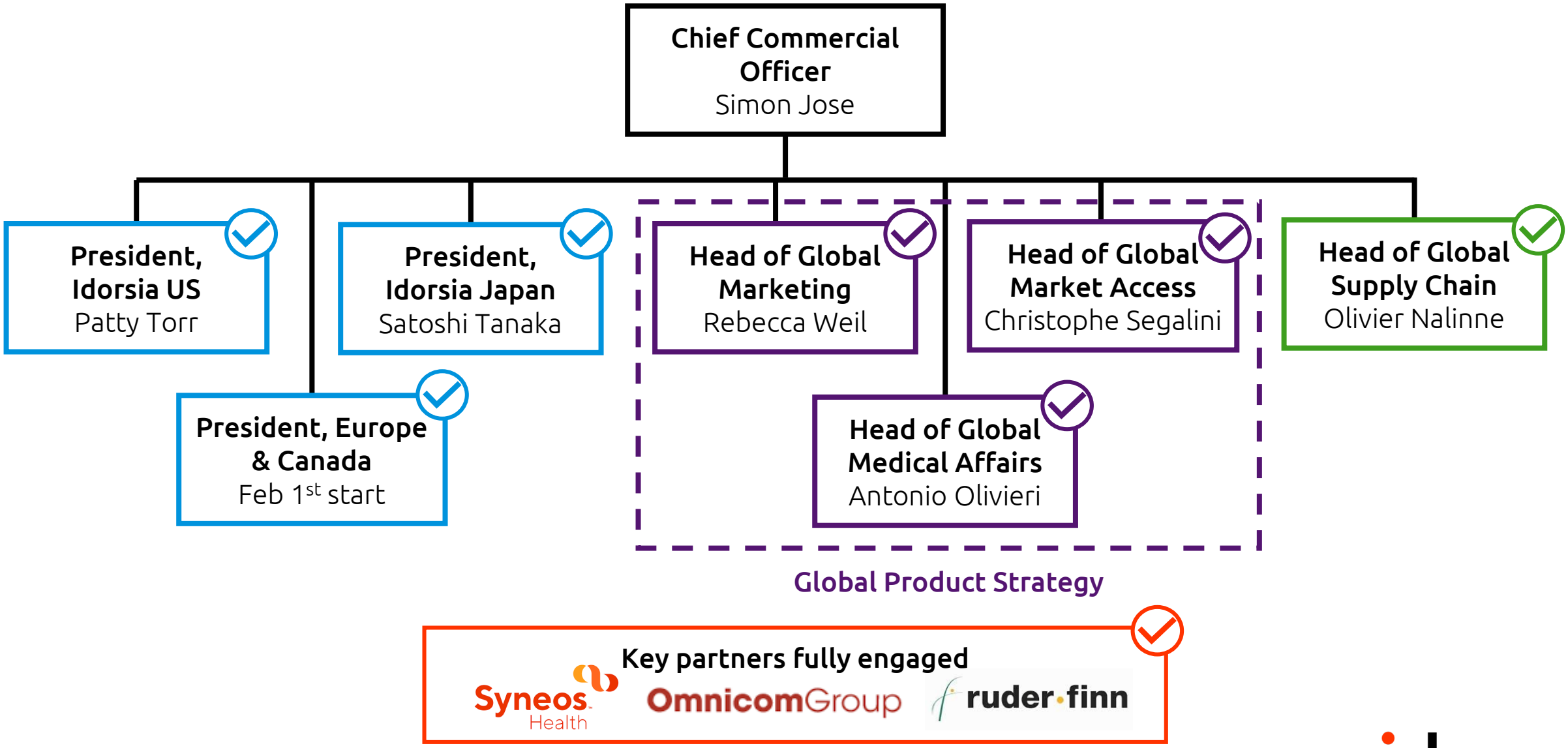
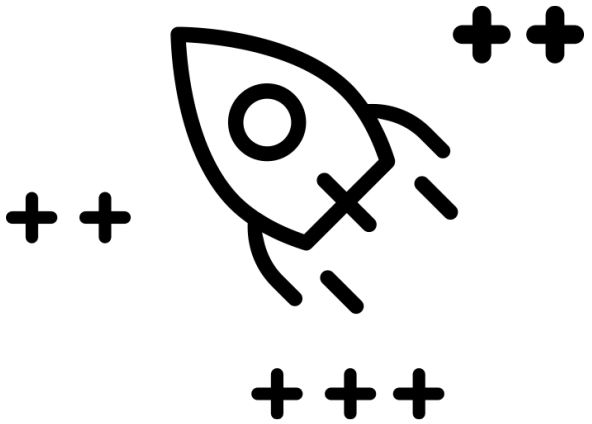
Idorsia is at an inflection point with major catalysts expected in the near-term



Building a commercial organization



2021 will be a key year for Idorsia



2021

Enter 2021 with a **strong balance sheet**
(liquidity + undrawn credit facility from J&J)

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

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

Royalty streams

- Ponesimod
- Aprocitentan
- T-type calcium channel blocker

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

Jean-Paul Clozel
Chief Executive Officer

idorsia

Be prepared for more!