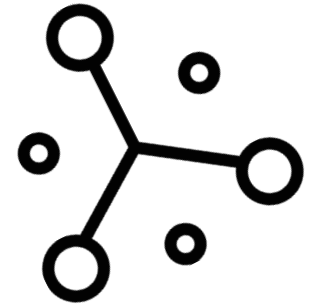
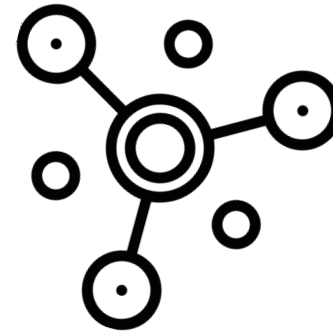
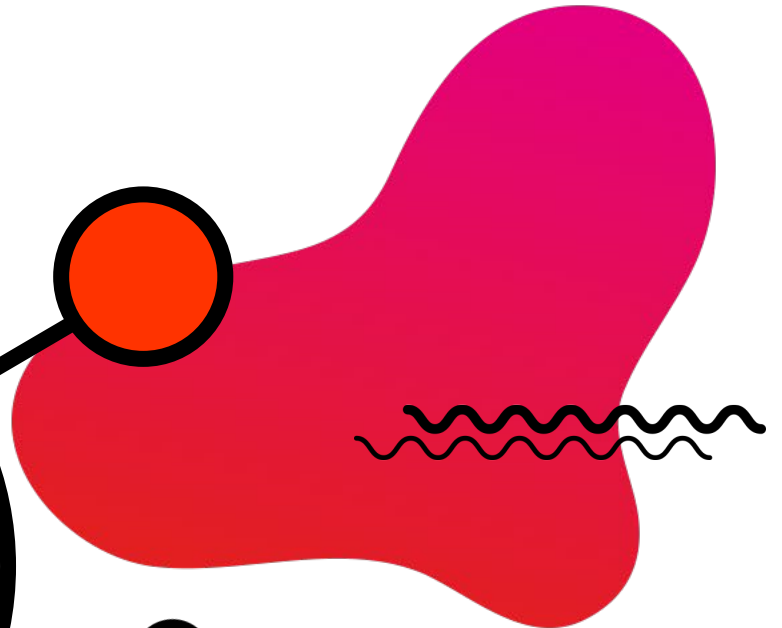
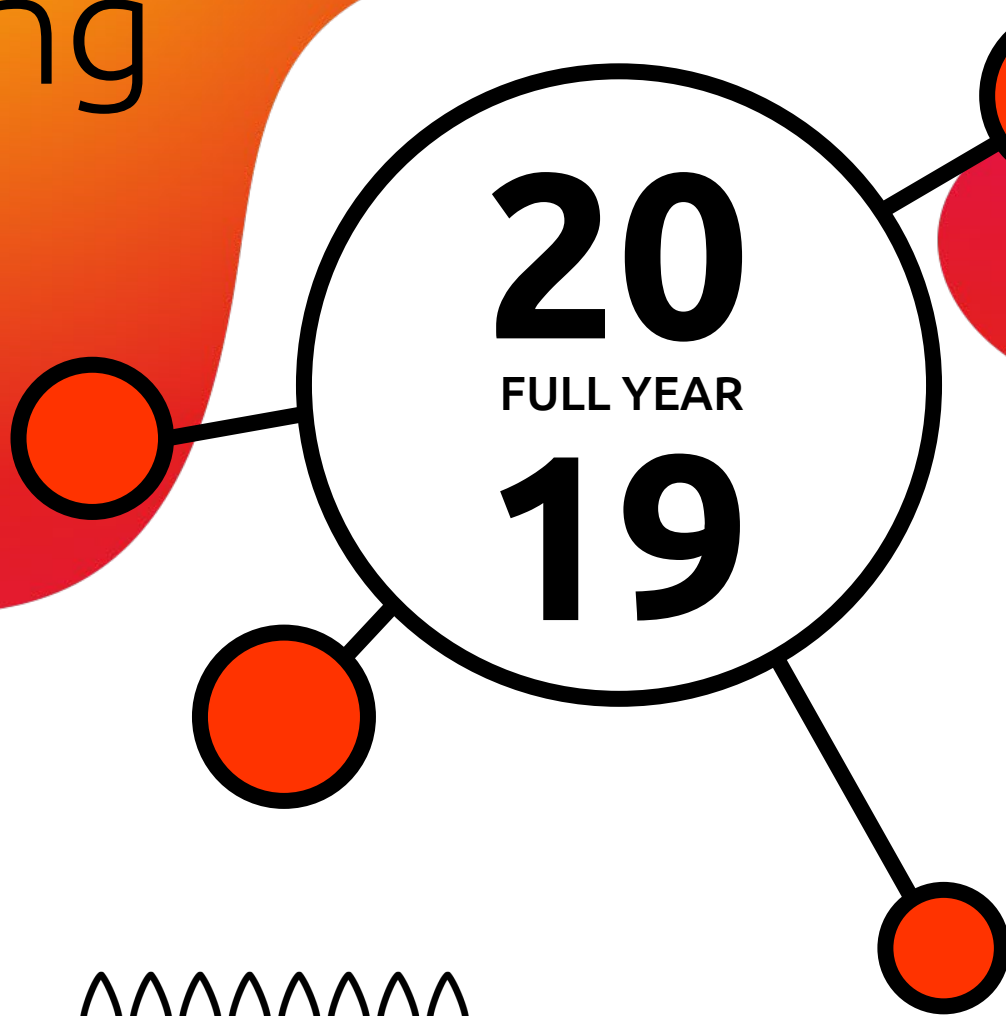
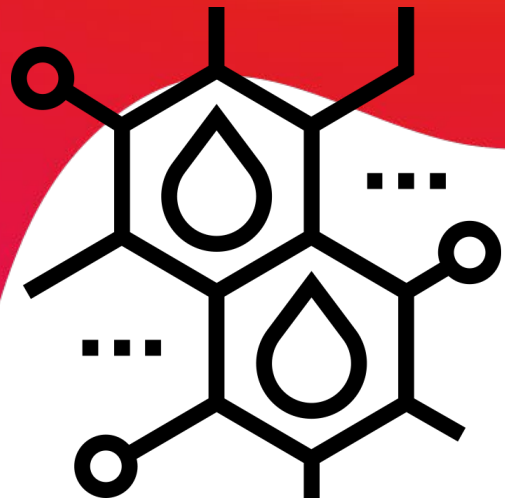


# Financial Reporting



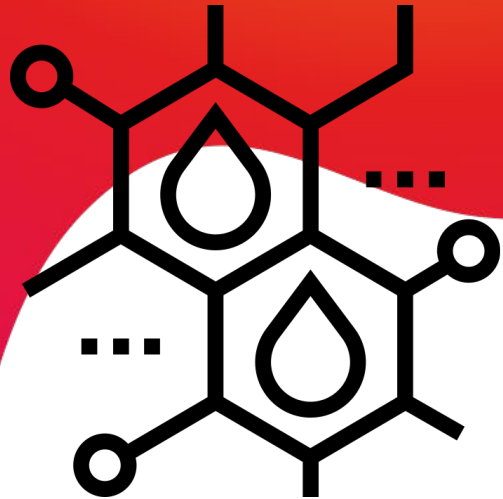
**idonesia**

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.

“Since the **results of the daridorexant program** are just months away, 2020 promises to be every bit as exciting as 2019.”

Jean-Paul Clozel  
Chief Executive Officer





# 2019 HIGHLIGHTS

Advancing our  
clinical programs

Engaging with the  
expert community

Delivering  
innovation

Building our  
commercial capabilities

# Clinical development pipeline

Compound	Mechanism of Action	Target Indication	Status
<b>Daridorexant</b>	Dual orexin receptor antagonist	Insomnia	Phase 3 – recruitment complete
<b>Aprocitentan*</b>	Dual endothelin receptor antagonist	Resistant hypertension management	Phase 3
<b>Clazosentan</b>	Endothelin receptor antagonist	Vasospasm associated with aneurysmal subarachnoid hemorrhage	Phase 3
<b>Lucerastat</b>	Glucosylceramide synthase inhibitor	Fabry disease	Phase 3
<b>Cenerimod</b>	S1P <sub>1</sub> receptor modulator	Systemic lupus erythematosus	Phase 2
<b>Selatogrel</b>	P2Y <sub>12</sub> receptor antagonist	Suspected acute myocardial infarction	Phase 2 – complete
<b>ACT-774312</b>	CRTH2 receptor antagonist	Nasal polyposis	Phase 2
<b>Sinbaglustat (ACT-519276)</b>	GBA2/GCS inhibitor	Rare CNS diseases	Phase 1
<b>ACT-539313</b>	Selective orexin 1 receptor antagonist	Psychiatric disorders	Phase 1
<b>ACT-709478**</b>	T-type calcium channel blocker	Epilepsy	Phase 1
<b>ACT-1004-1239</b>	-	Immunology / Cancer immunotherapy	Phase 1
<b>ACT-1014-6470</b>	-	Immunology	Phase 1

\* In collaboration with Janssen Biotech, Inc.

\*\* Idorsia has granted to Neurocrine Biosciences, Inc. an option to license ACT-709478, this option will expire 30 days after the IND application acceptance by the FDA, expected in mid-2020

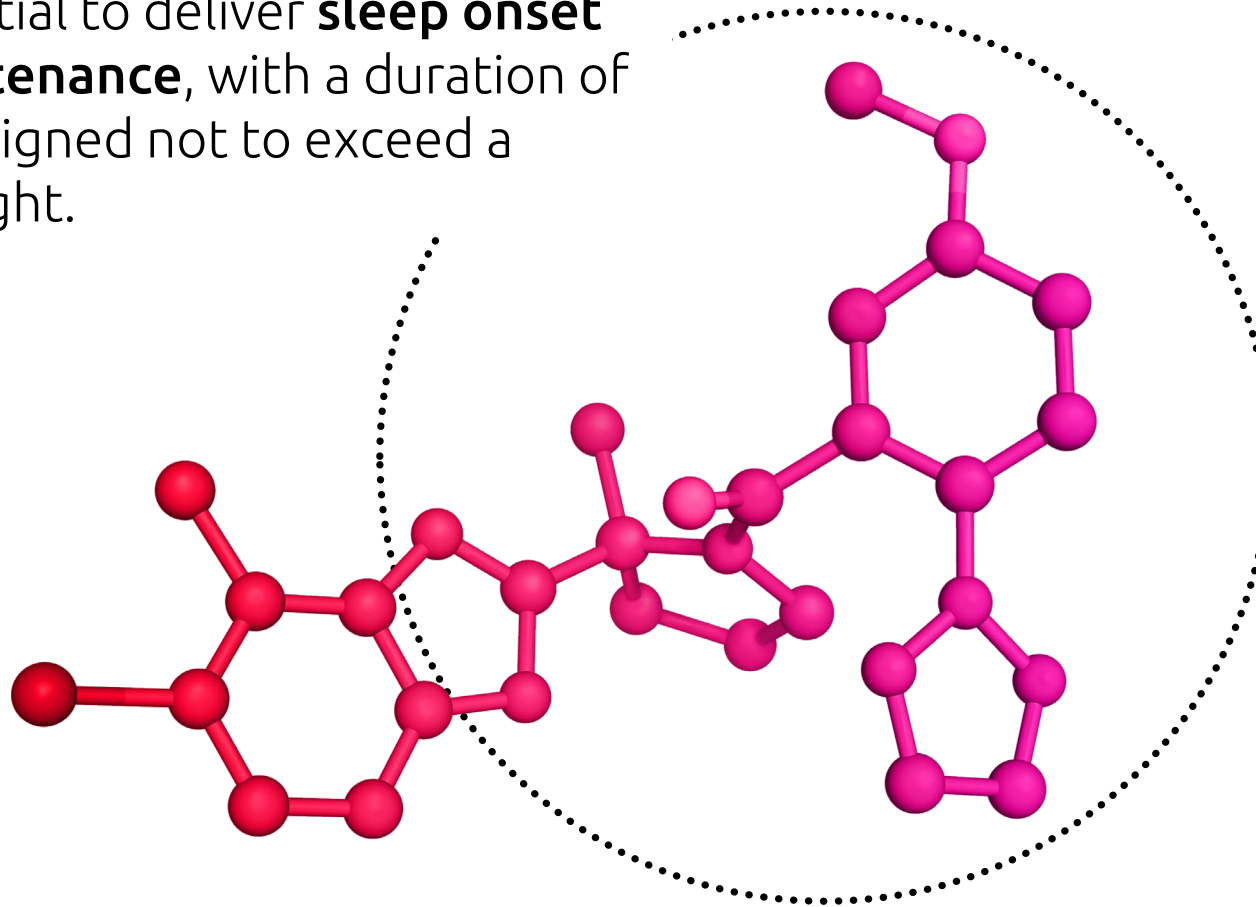
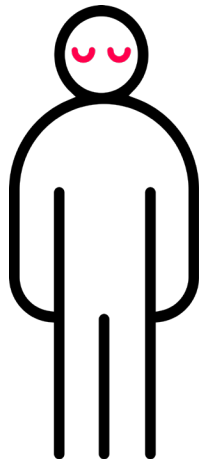
# Late-stage assets

Compound	Mechanism of Action	Target Indication	Status
<b>Daridorexant</b>	Dual orexin receptor antagonist	Insomnia	Phase 3 – recruitment complete
<b>Aprocitentan*</b>	Dual endothelin receptor antagonist	Resistant hypertension management	Phase 3
<b>Clazosentan</b>	Endothelin receptor antagonist	Vasospasm associated with aneurysmal subarachnoid hemorrhage	Phase 3
<b>Lucerastat</b>	Glucosylceramide synthase inhibitor	Fabry disease	Phase 3

\* In collaboration with Janssen Biotech, Inc.

# Daridorexant in insomnia

A dual orexin receptor antagonist with the potential to deliver **sleep onset and maintenance**, with a duration of action designed not to exceed a normal night.



## Phase 2 data presented

- Oral and poster presentations at the **annual SLEEP meeting 2019** attended by around **5,000** delegates
- Poster presentations at **World Sleep 2019**, with over **3,300** attendees

## Phase 3 results expected soon

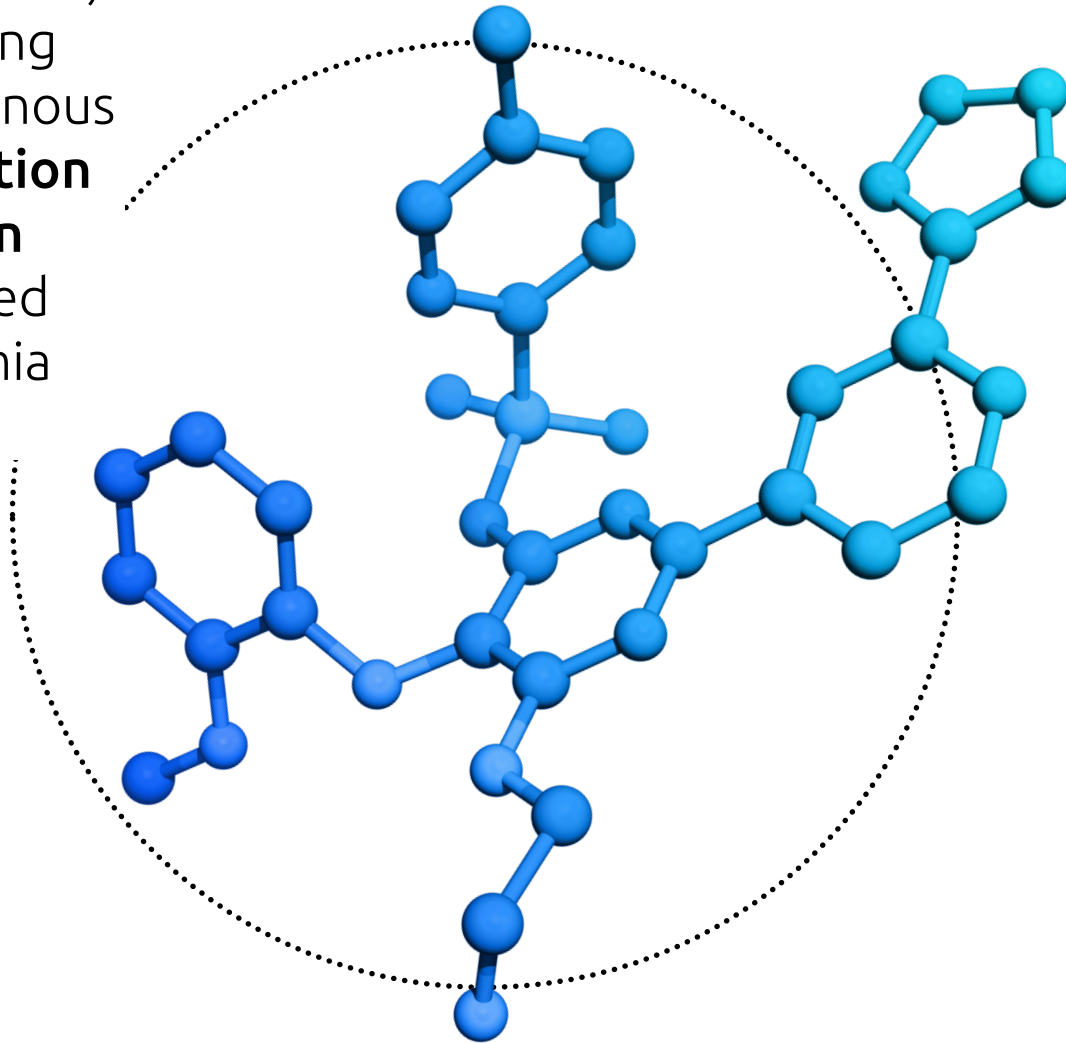
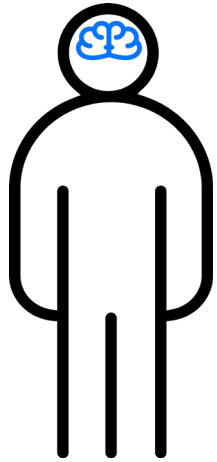
- Assessing efficacy **during the night**, impact on patient's functioning **during the day**
- First Phase 3 study (25 and 50mg) in around 900 adult and elderly patients with insomnia to **read out in Q2 2020**

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



# Clazosentan in cerebral vasospasm

A selective endothelin (ETA) receptor antagonist being developed as an intravenous infusion for the **prevention of clinical deterioration** due to vasospasm-related delayed cerebral ischemia following aSAH



## Registration studies in Japan

- Evaluating reduction of vasospasm, and vasospasm-related morbidity and mortality following aSAH
- Results expected in 2H 2020

## Global Phase 3 study ongoing

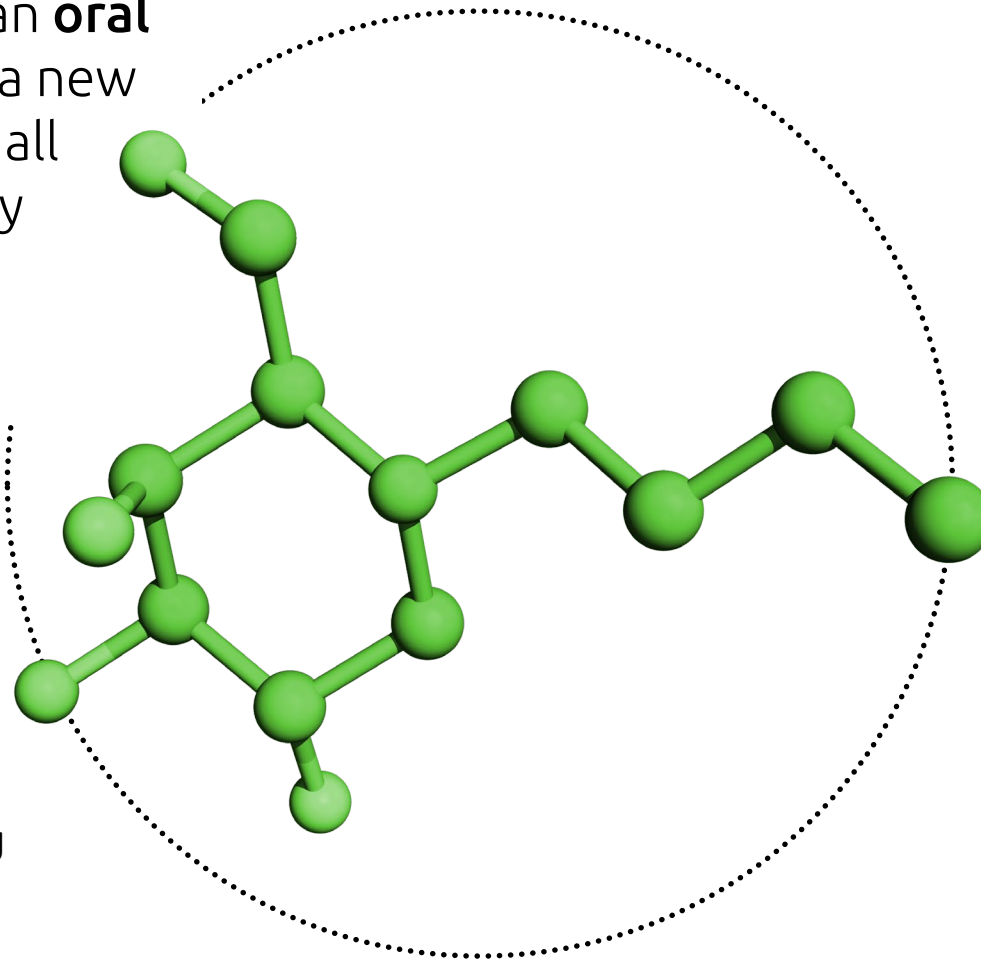
- REACT – investigating the efficacy and safety of clazosentan in an enriched aSAH population
- Commenced enrollment in early 2019

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



# Lucerastat in Fabry disease

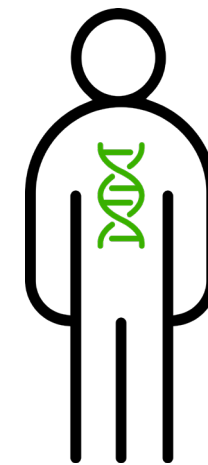
A glucosylceramide synthase inhibitor, developed as an **oral monotherapy**, offering a new treatment approach for all patients living with Fabry disease, **irrespective of mutation type**



Lucerastat has **orphan drug** status in US and EU

## Phase 3 study ongoing

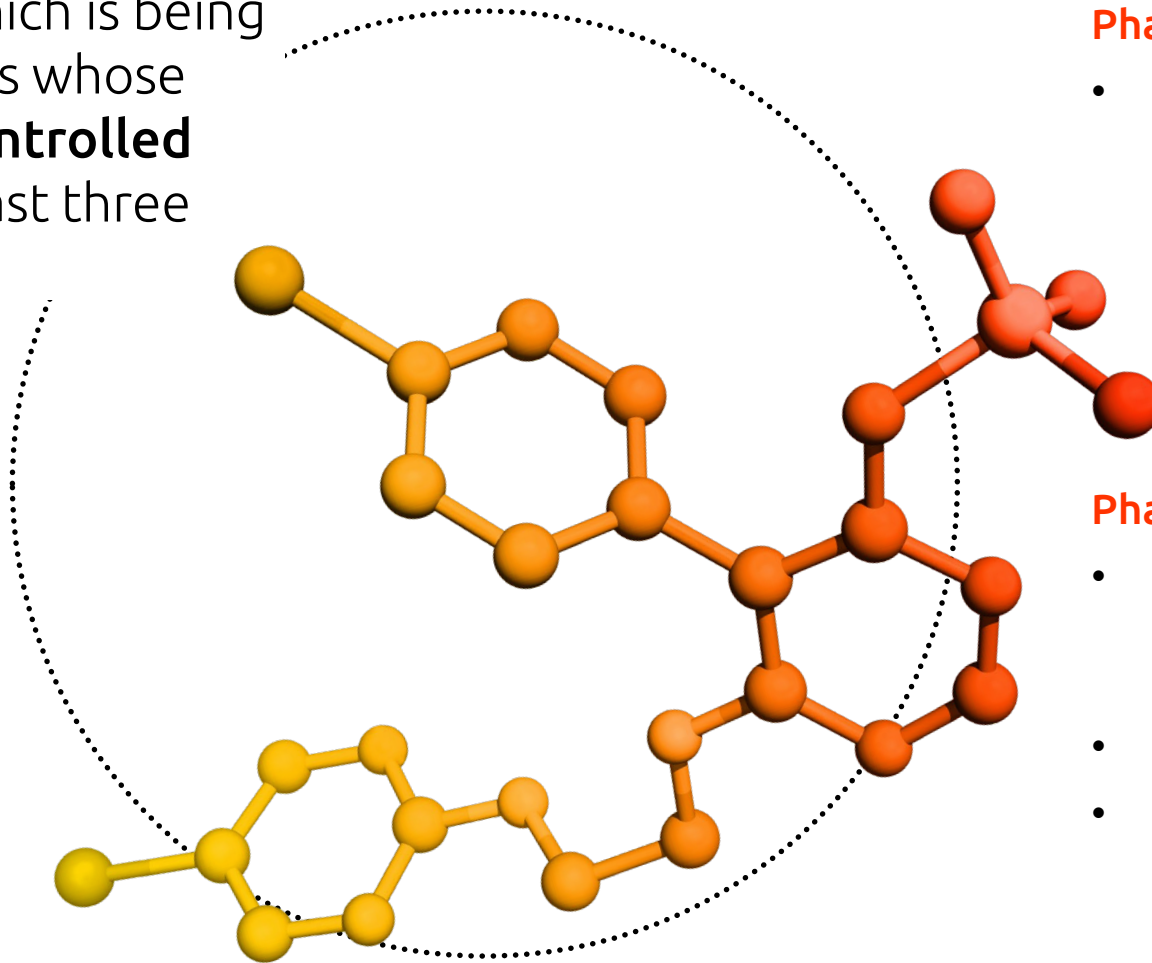
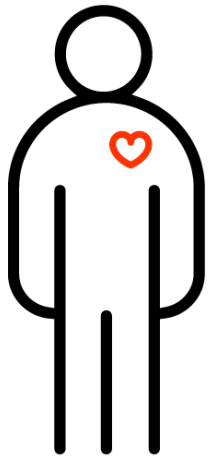
- Lucerastat acts by reducing the damaging build-up of lipids which is responsible for all the symptoms of Fabry disease
- MODIFY – to assess effects of lucerastat on **neuropathic pain**, a symptom having a severe impact on many patients' lives
- The study is expected to report **results towards mid-2021**



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

# Aprocitentan in resistant hypertension

An orally active dual endothelin receptor antagonist, which is being investigated for patients whose **blood pressure is uncontrolled** despite the use of at least three antihypertensive drugs



## Phase 2 data presented

- Oral presentation at the European Society of Cardiology (**ESC Congress 2019**), attended by almost **35,000** experts

## Phase 3 study ongoing

- Evaluating **initial and long-term effects** of aprocitentan **on systolic and diastolic blood pressure**
- **Initiated in June 2018**
- Collaborating with **Janssen Biotech** to jointly develop and commercialize aprocitentan

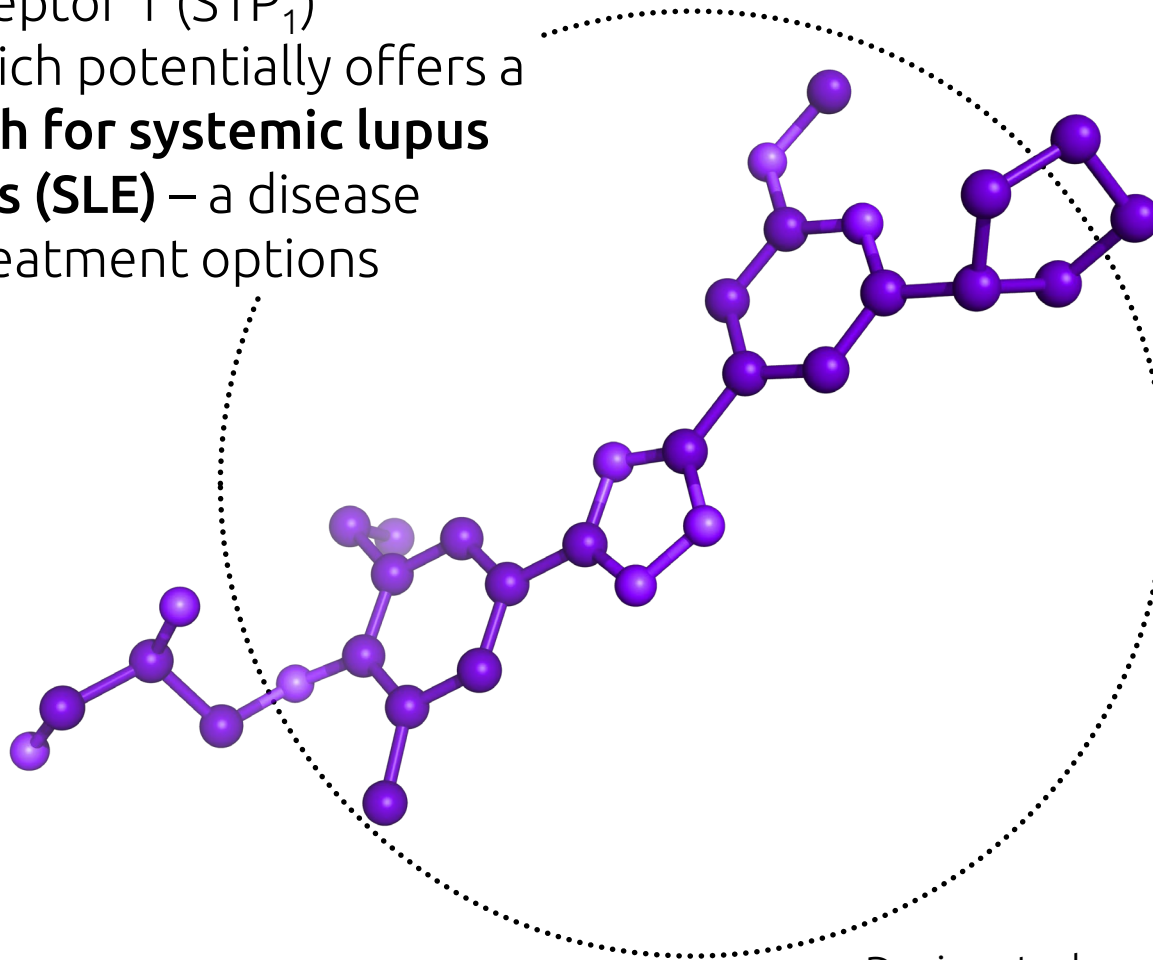
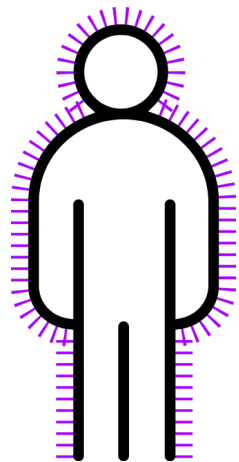
Aprocitentan is investigational, in development and not approved or marketed in any country.

# Mid-stage assets

Compound	Mechanism of Action	Target Indication	Status
<b>Cenerimod</b>	S1P <sub>1</sub> receptor modulator	Systemic lupus erythematosus	Phase 2
<b>Selatogrel</b>	P2Y <sub>12</sub> receptor antagonist	Suspected acute myocardial infarction	Phase 2 – complete
<b>ACT-774312</b>	CRTH2 receptor antagonist	Nasal polyposis	Phase 2

# Cenerimod in SLE

A potent, selective sphingosine-1-phosphate receptor 1 (S1P<sub>1</sub>) modulator, which potentially offers a **novel approach for systemic lupus erythematosus (SLE)** – a disease with limited treatment options



## Phase 2 data presented

- Oral and poster presentations at the annual meeting 2019 of the **American College of Rheumatology**, with over **15,000** delegates

## Multiple-dose efficacy and safety study

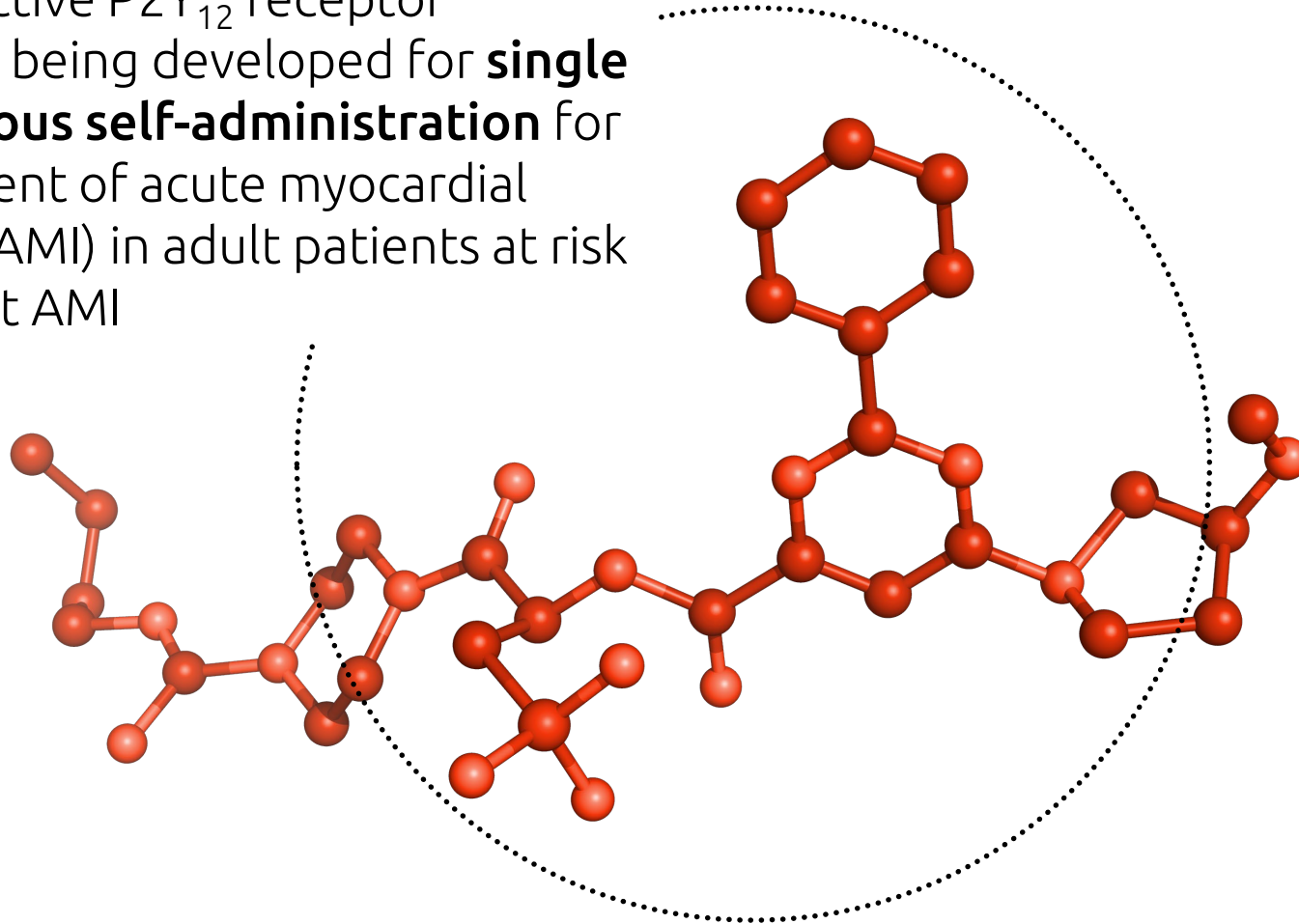
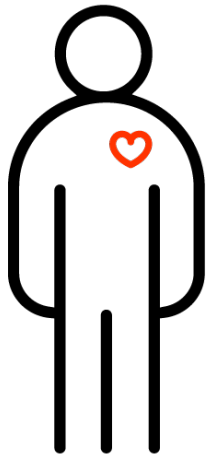
- Evaluating the treatment of adult patients with moderately to severely active, autoantibody-positive systemic lupus erythematosus
- **Initiated in January 2019**

Designated as a **Fast Track development program** by the US FDA

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

# Selatogrel to prevent AMI

A potent, fast-acting, reversible, and highly-selective P2Y<sub>12</sub> receptor antagonist, being developed for **single subcutaneous self-administration** for the treatment of acute myocardial infarction (AMI) in adult patients at risk of recurrent AMI



## Phase 2 data presented

- Oral presentations at the European Society of Cardiology (**ESC Congress 2019**), attended by almost **35,000** experts

## Phase 3 study in preparation

- Global agreement with **Antares Pharma** to develop a novel drug-device product
- Product is currently being tested through **usability and reliability studies** tailored for emergency use
- Phase 3 study to be initiated in **1H 2021**

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

# Early-stage assets

Compound	Mechanism of Action	Target Indication	Status
<b>Sinbaglustat (ACT-519276)</b>	GBA2/GCS inhibitor	Rare CNS diseases	Phase 1
<b>ACT-539313</b>	Selective orexin 1 receptor antagonist	Psychiatric disorders	Phase 1
<b>ACT-709478**</b>	T-type calcium channel blocker	Epilepsy	Phase 1
<b>ACT-1004-1239</b>	-	Immunology / Cancer immunotherapy	Phase 1
<b>ACT-1014-6470</b>	-	Immunology	Phase 1

\*\* Idorsia has granted to Neurocrine Biosciences, Inc. an option to license ACT-709478, this option will expire 30 days after the IND application acceptance by the FDA, expected in mid-2020



# Engaging with the expert community

American College of Rheumatology (ACR)  
Annual meeting 2019

European Society of Cardiology (ESC)  
ESC Congress 2019

American Academy of Sleep Medicine (AASM) / Sleep Research Society (SRS)  
Annual SLEEP meeting 2019

World Sleep Society  
World Sleep 2019

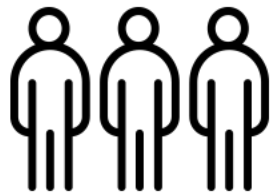
European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS)  
Annual international conference 2019

**>60,000 experts**

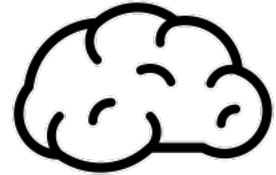




# Building a bright future



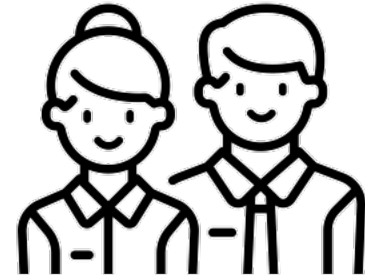
**>800**  
employees



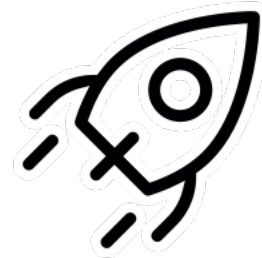
**Highly**  
qualified  
professionals



**34**  
nationalities



**43%** **57%**  
female male



**One**  
common goal



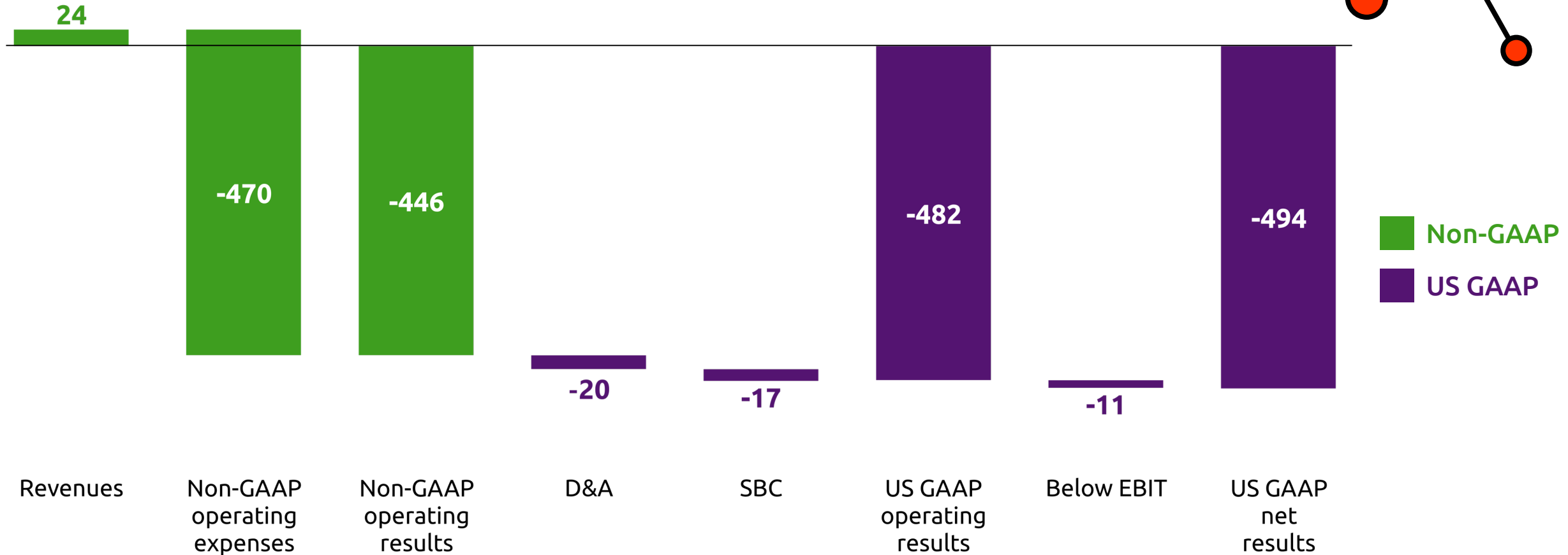
“With a cost-conscious attitude and a slight shift in timelines, we spent less in 2019 than originally expected.”

**André C. Muller**  
**Chief Financial Officer**



# US GAAP net results

in CHF millions, rounding differences may occur

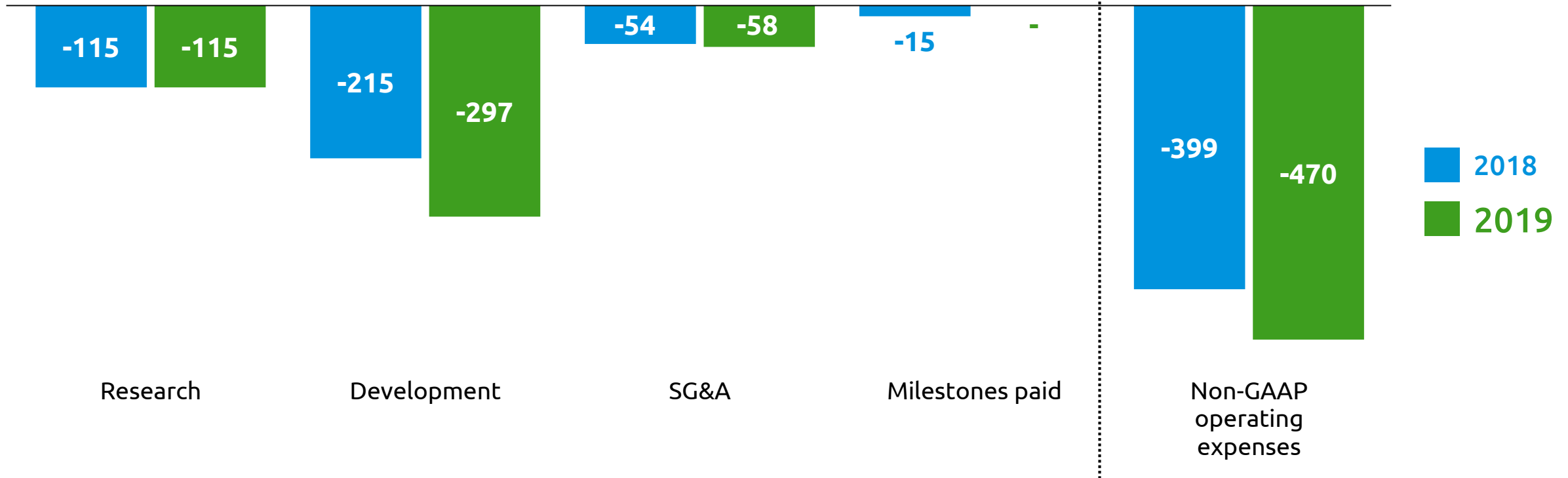
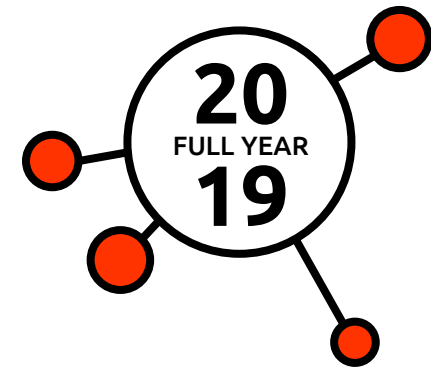


Financial results as of Dec 31, 2019



# Non-GAAP operating expenses

in CHF millions, rounding differences may occur

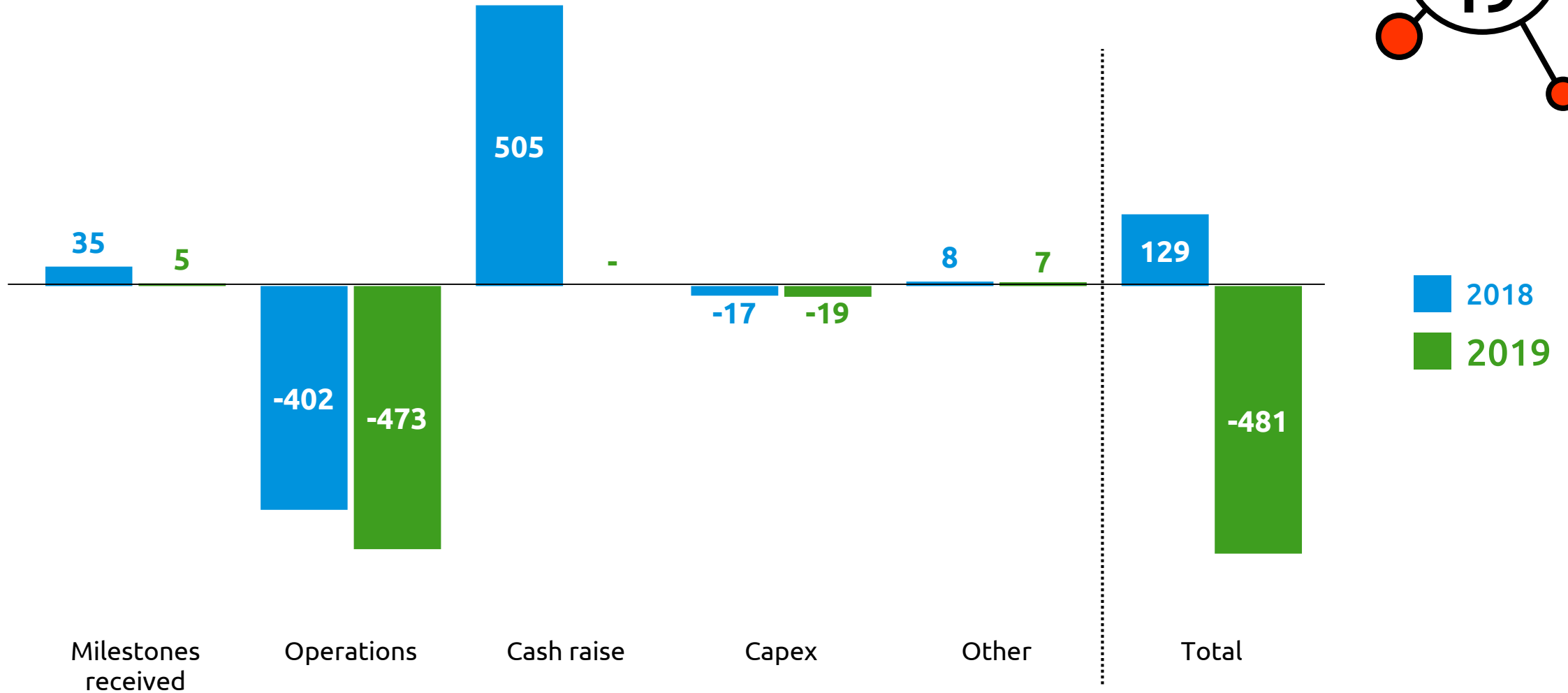
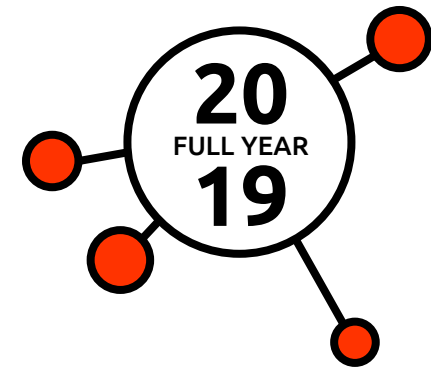


Financial results as of Dec 31, 2019



# Cash flow

in CHF millions, rounding differences may occur

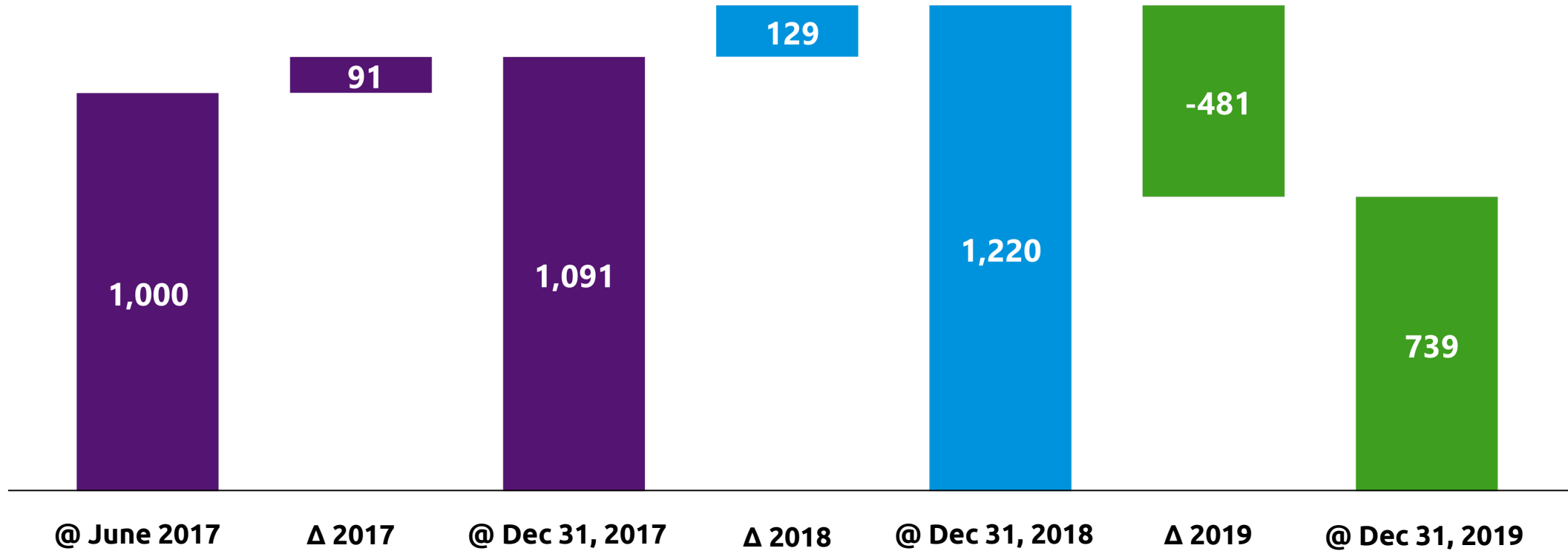
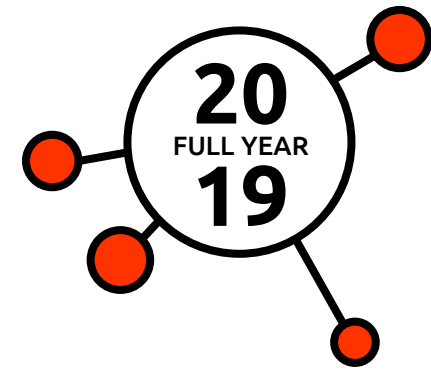


Financial results as of Dec 31, 2019



# Liquidity

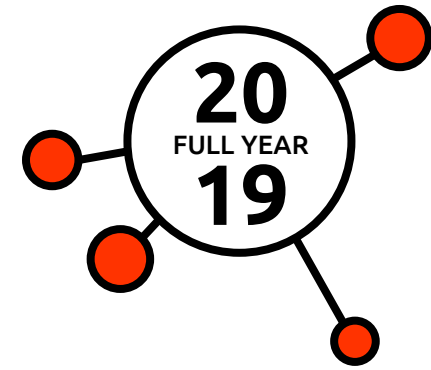
in CHF millions, rounding differences may occur



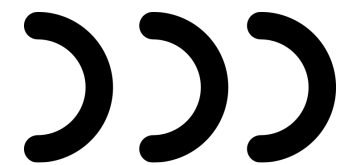
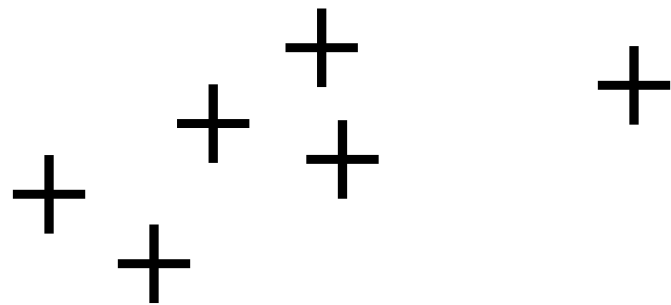
Financial results as of Dec 31, 2019



# Financial Guidance for 2020



Unforeseen events excluded, US GAAP operating expenses of around CHF 540 million and non-GAAP operating expenses of around CHF 500 million (both measures excluding any potential milestone payments).





“Since the **results of the daridorexant program** are just months away, 2020 promises to be every bit as exciting as 2019.”

Jean-Paul Clozel  
Chief Executive Officer

