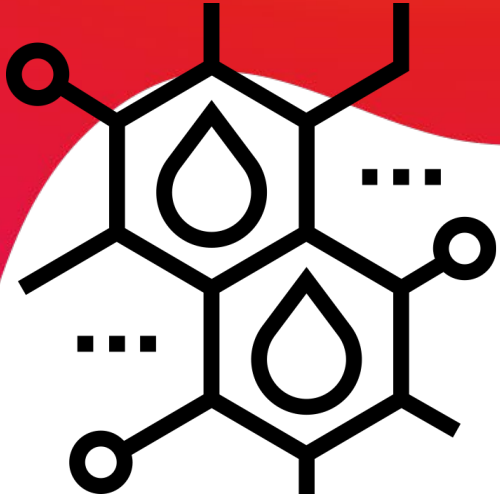


# Financial Reporting



**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.

“The highlight in the first half of 2020 is the positive daridorexant results – we are now preparing to file with the US FDA around the end of this year.”

Jean-Paul Clozel  
Chief Executive Officer





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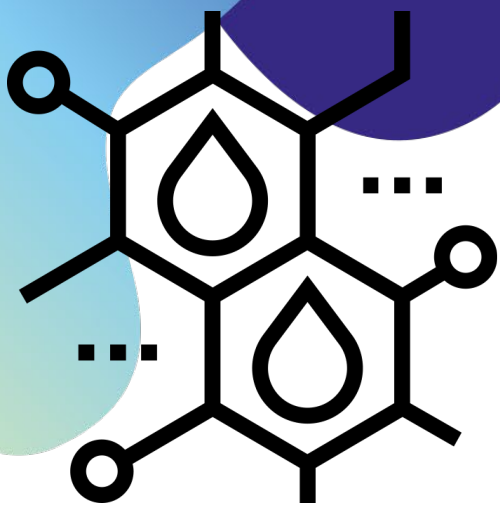
HALF YEAR

20

Positive pivotal results with daridorexant

License agreement with Neurocrine

Issuance of 11 million new shares raises CHF 330 million



Janssen submitted NDA and EMA for ponesimod

# Clinical development pipeline

Compound	Mechanism of Action	Target Indication	Status
<b>Daridorexant</b>	Dual orexin receptor antagonist	Insomnia	Filing in preparation
<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>Selatogrel</b>	P2Y <sub>12</sub> receptor antagonist	Suspected acute myocardial infarction	Phase 3 in preparation
<b>Cenerimod</b>	S1P <sub>1</sub> receptor modulator	Systemic lupus erythematosus	Phase 2
<b>ACT-774312</b>	CRTH2 receptor antagonist	Nasal polyposis	Phase 2
<b>ACT-539313</b>	Selective orexin 1 receptor antagonist	Psychiatric disorders	Phase 2 in preparation
<b>Sinbaglustat</b>	GBA2/GCS inhibitor	Rare lysosomal storage disorders	Phase 1 complete
<b>ACT-1004-1239</b>	-	Immunology / Cancer immunotherapy	Phase 1
<b>ACT-1014-6470</b>	-	Immunology	Phase 1
<b>ACT-541478</b>	-	CNS	Phase 1

\* In collaboration with Janssen Biotech to jointly develop and solely commercialize Idorsia's aprocitentan worldwide

- Neurocrine Biosciences has a global license to develop and commercialize Idorsia's ACT-709478, a novel T-type calcium channel blocker, for the treatment of a rare form of pediatric epilepsy. A Phase 2 study is planned for the second half of 2020.
- Idorsia has the option to license vamorolone from ReveraGen Inc. and has granted to Santhera Holding Ltd the option to sub-license vamorolone worldwide (except Japan and South-Korea) for all indications.

# Our Strategic Priorities

Our mid-term key priorities to achieve long-term success:

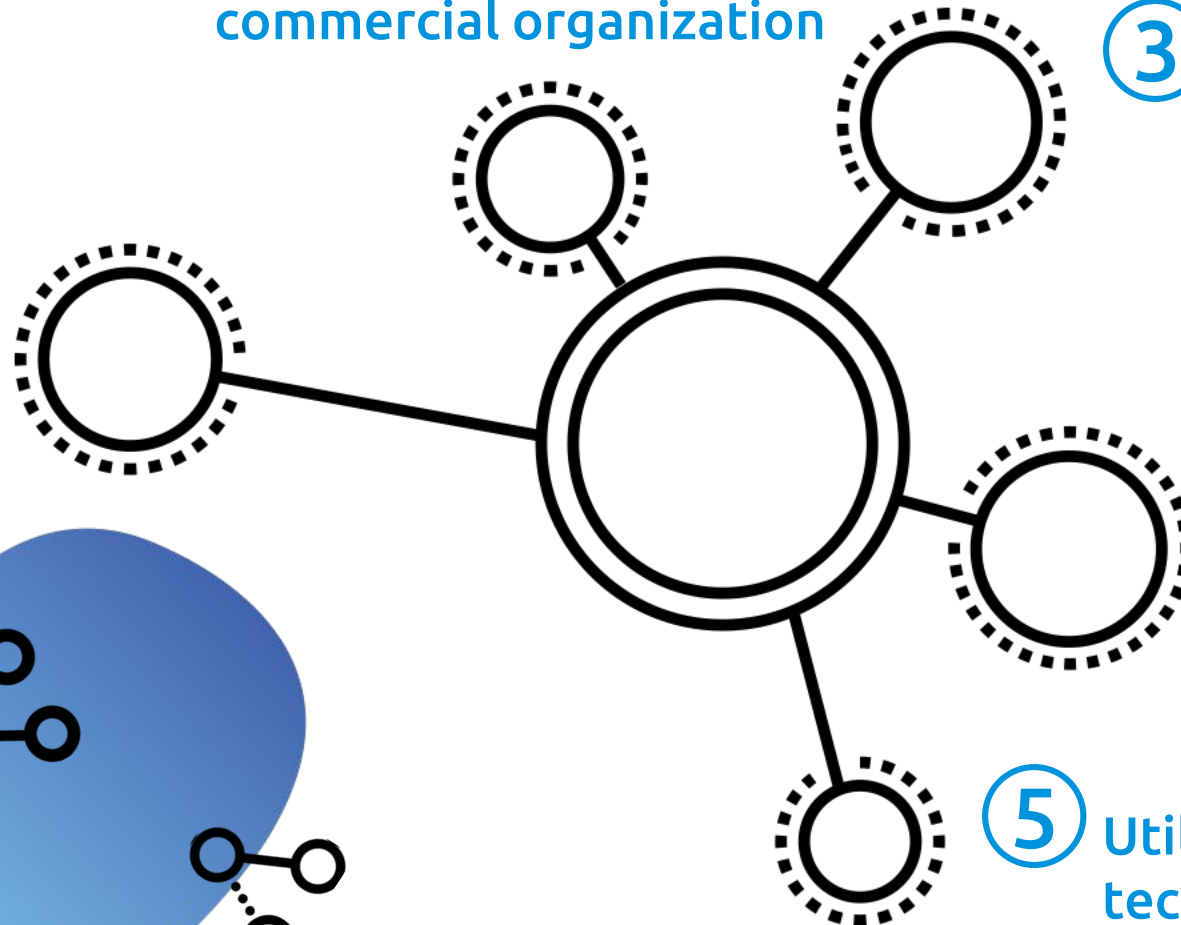
**1** Deliver at least three products to market

**2** Build and integrate a commercial organization

**3** Bring Idorsia to sustainable profitability

**4** Create a pipeline with a sales potential of CHF 5 billion

**5** Utilize state-of-the-art technologies

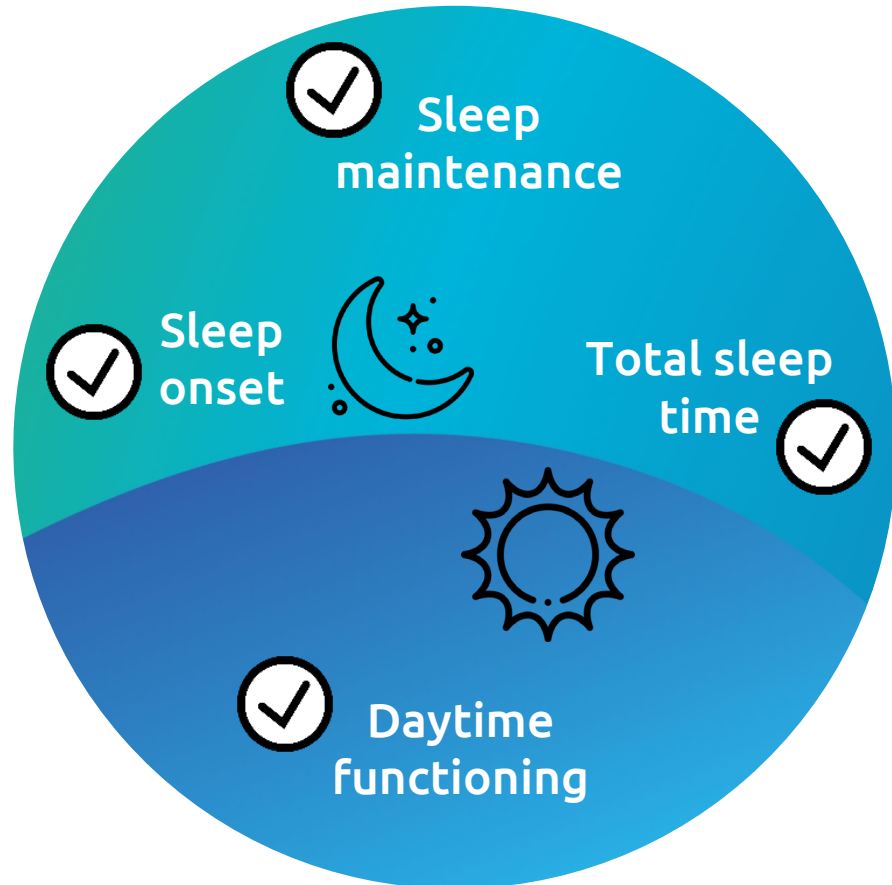




# Daridorexant – Pivotal program

The program with daridorexant demonstrated statistically significant and clinically meaningful improvements at month 1 and at month 3

## 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

“We are moving at full power following the outstanding results with daridorexant.”

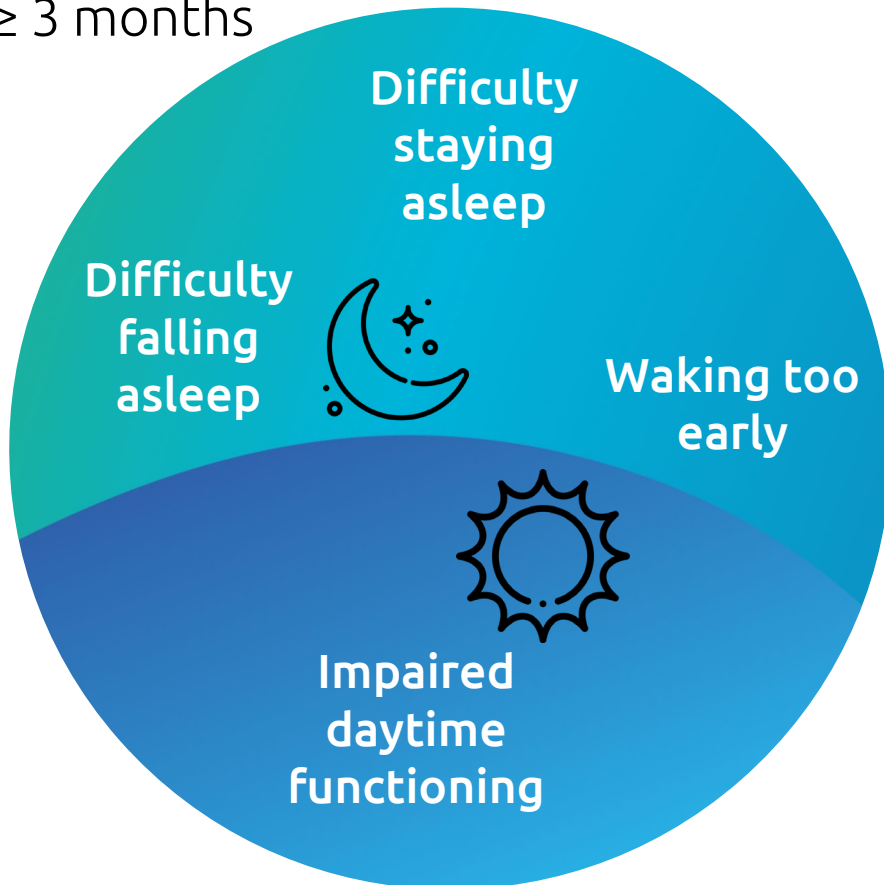
**Simon Jose**  
**Chief Commercial Officer**





# Insomnia impacts millions of people during the night and the day

≥ 3 nights/week  
≥ 3 months



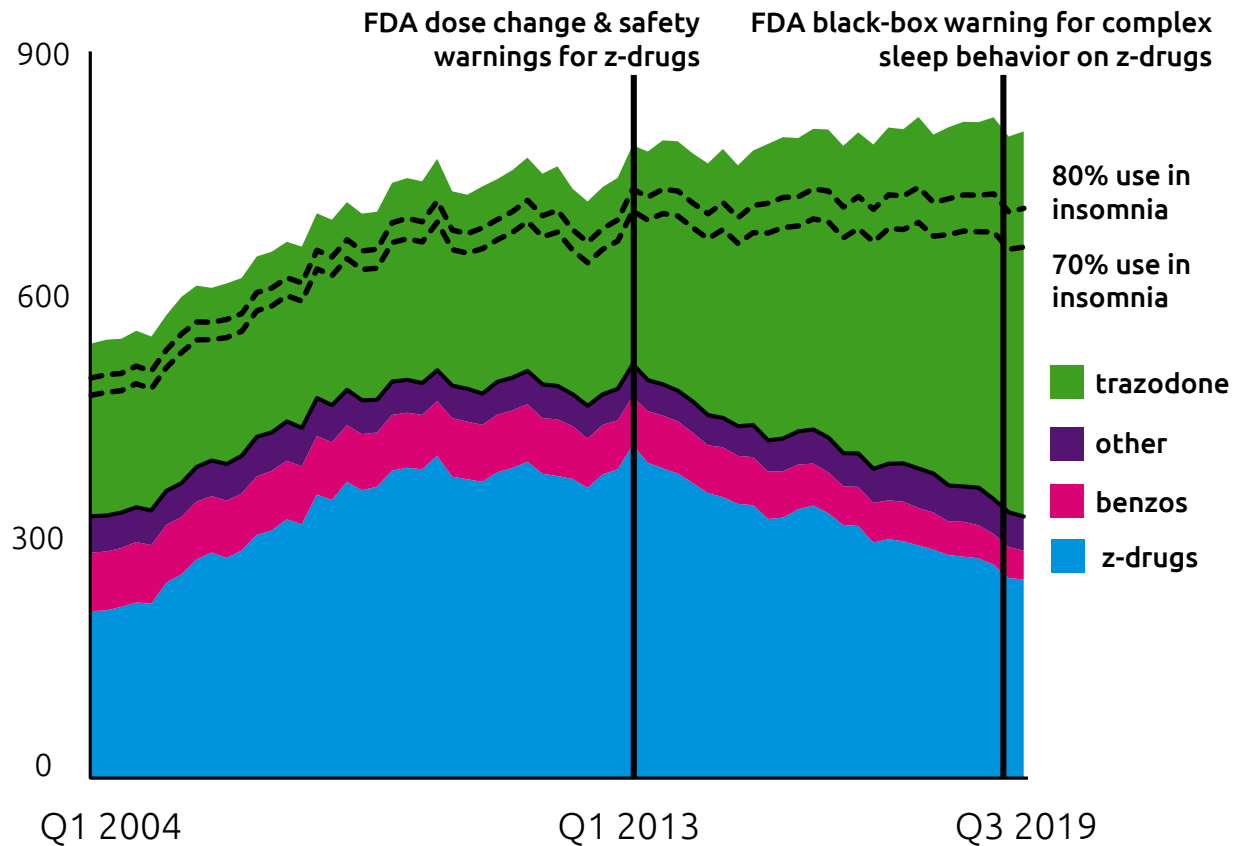
- Estimated 20 million<sup>1</sup> adults in the US suffer from chronic insomnia
- Insomnia has a significant impact on patients' productivity, quality of life and long-term health outcomes
- Existing therapies typically improve **either** onset or maintenance, have not demonstrated benefit on daytime performance and are associated with well known adverse events

1 Morin CM, et al. Insomnia disorder. Nat Rev Dis Primers 2015;1:15026

# High unmet need for effective, safe medications

## US prescription insomnia market

Quarterly Standard units (millions)<sup>1</sup>



- Decline in z-drug prescriptions since FDA issued warnings in 2013
- Corresponding increase in trazodone prescriptions over same time period
  - Estimated 70-80% of trazodone prescriptions are at doses commonly used for insomnia treatment<sup>2</sup>
- AASM guidelines recommend against use of trazodone to treat insomnia<sup>3</sup>

<sup>1</sup> IQVIA MIDAS quarterly standard units Q1 2004 – Q1 2019

<sup>2</sup> Symphony Health Solutions; Wong et al, BMJ 2017; 356:j603

<sup>3</sup> Sateia et al., Journal of Clinical Sleep Medicine 2017; 13(2):307-349

# Preparing for a successful launch

Opportunity to transform and modernize insomnia market

**Disruptive**

**Differentiated**

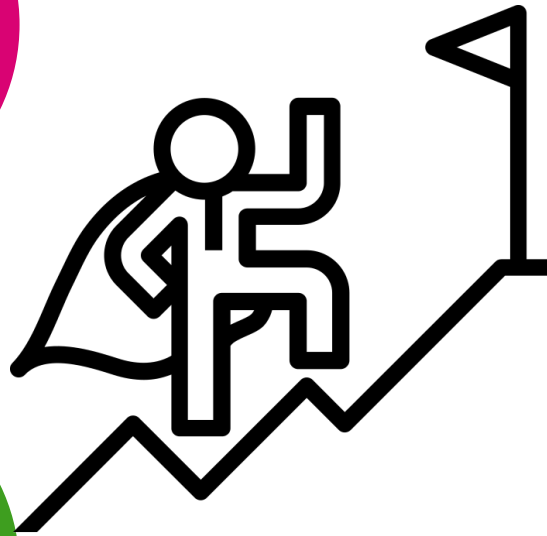
Potential to improve sleep **and** daytime performance without compromising safety

Strong regional partners to reach broad primary care audience

**Smart partnering**

**Nimble**

Simple, efficient approach to building core commercial capabilities



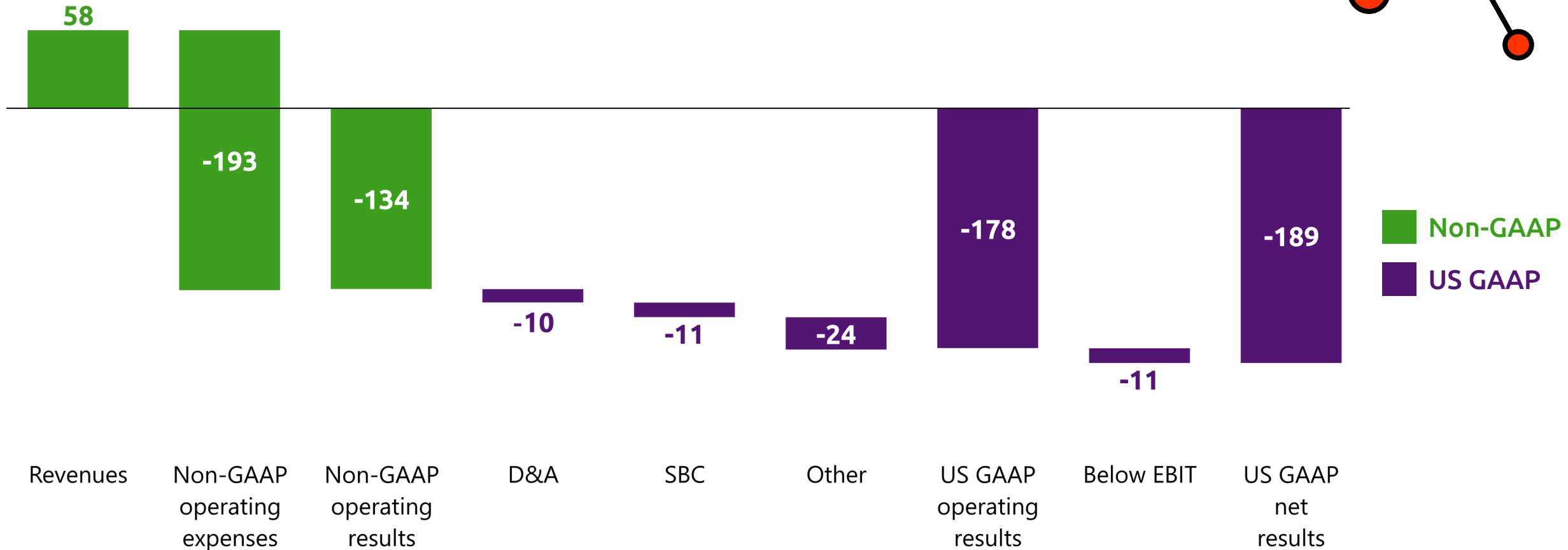
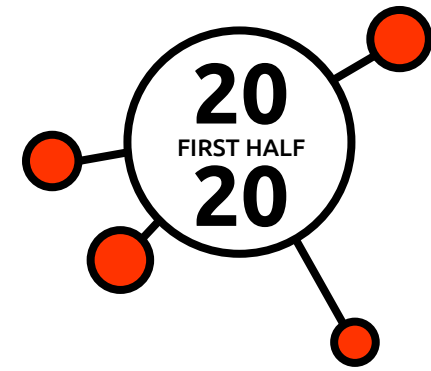
“Cost-control and the COVID-19 pandemic have resulted in lower than expected OPEX.”

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



# US GAAP net results

in CHF millions, rounding differences may occur



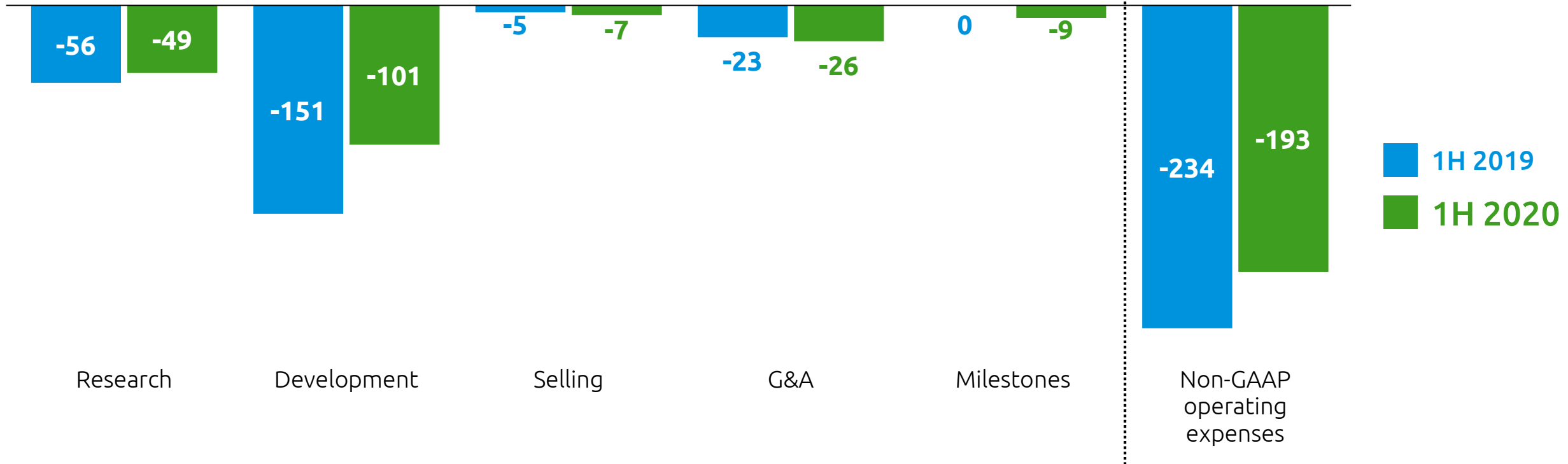
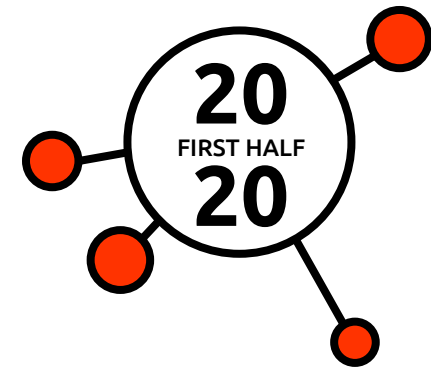
Financial results as of June 30, 2020





# Non-GAAP operating expenses

in CHF millions, rounding differences may occur

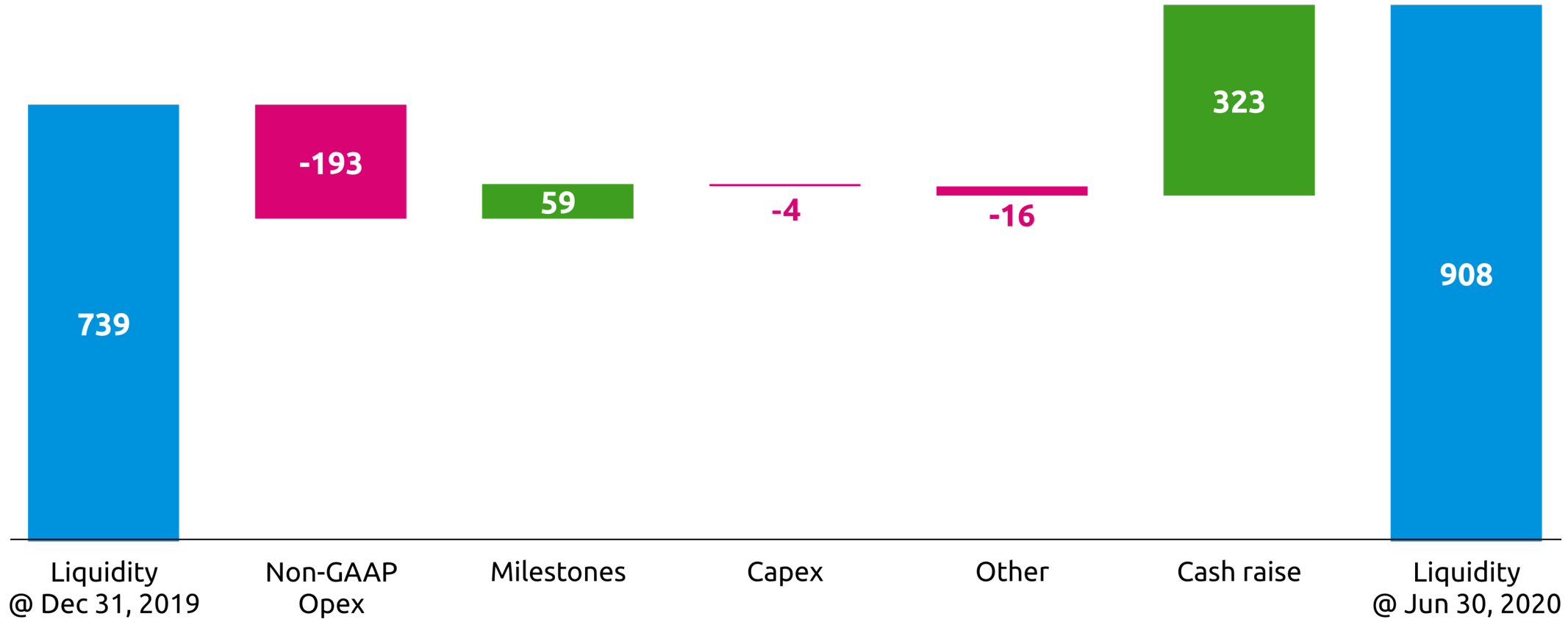
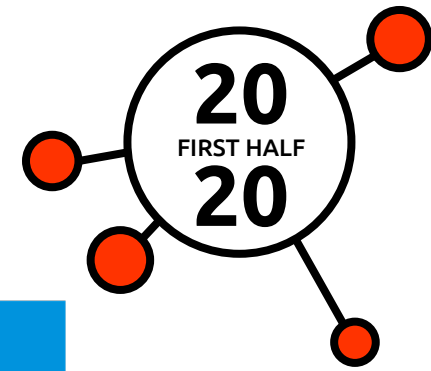


Financial results as of June 30, 2020



# Cash flow

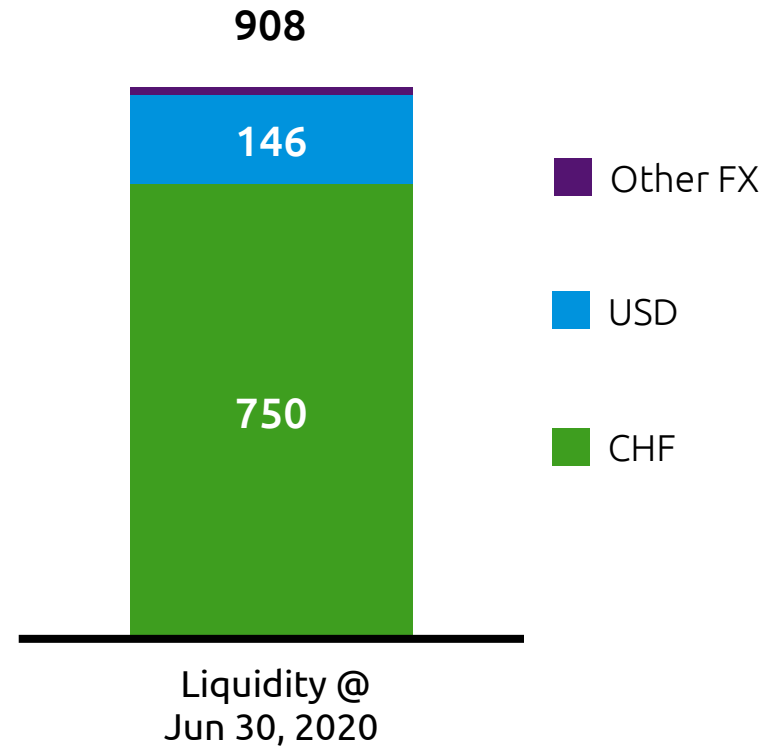
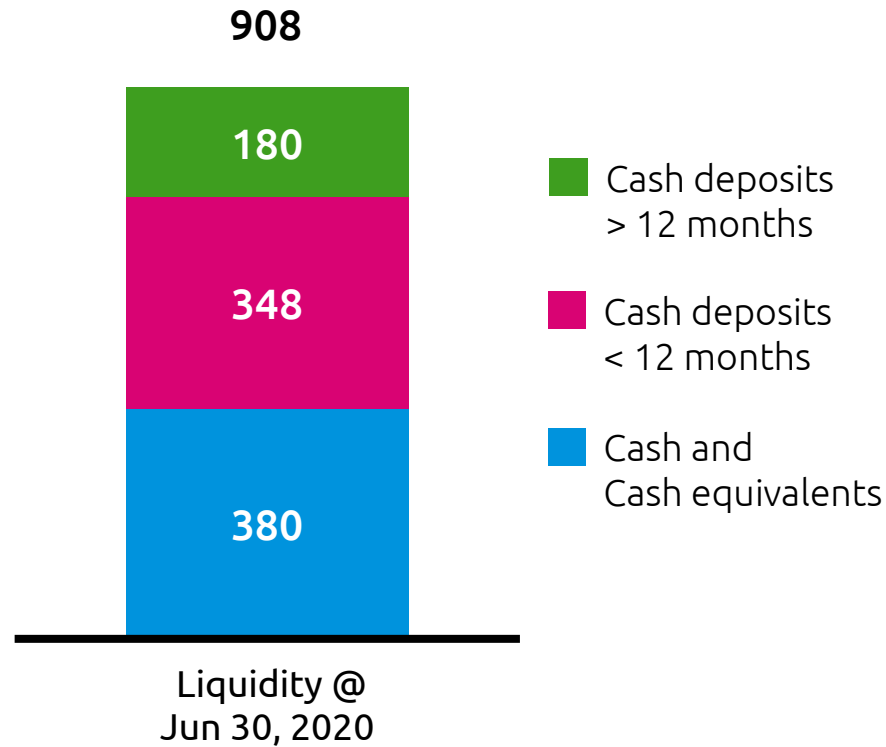
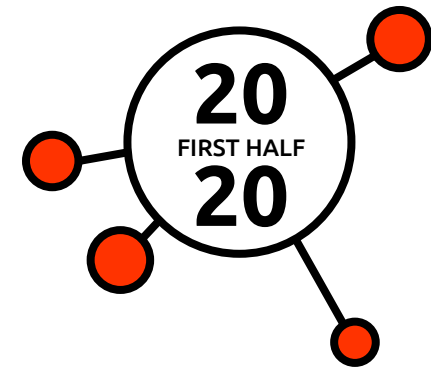
in CHF millions, rounding differences may occur



Financial results as of June 30, 2020

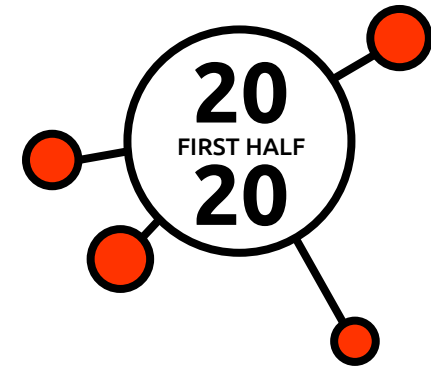
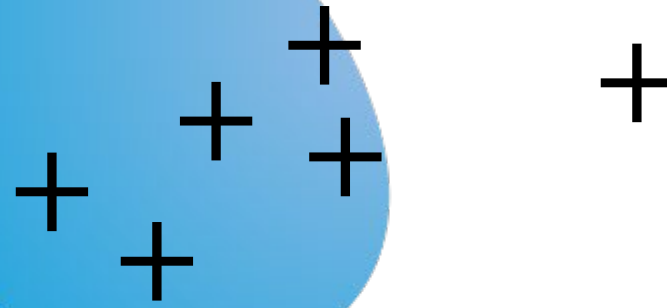
# Liquidity

in CHF millions, rounding differences may occur



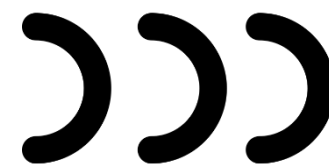
Financial results as of June 30, 2020

# Financial Guidance for 2020



US GAAP operating expenses around CHF 530 million and non-GAAP operating expenses around CHF 490 million

Both measures exclude unforeseen events, potential milestone payments and any potential award granted in the ongoing arbitration



# Stay tuned!

“The second half of 2020 promises to be every bit as exciting as the first.”

Jean-Paul Clozel  
Chief Executive Officer





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