Idorsia Company Profile

Headquartered in Switzerland – a biotech-hub of Europe – Idorsia is a high-potential biopharmaceutical company, specialized in the discovery, development and commercialization of innovative small molecules, with the aim of transforming the horizon of therapeutic options. The company has an experienced team of over 1,200 highly qualified professionals, a full R&D pipeline, state-of-the-art facilities, and a strong balance sheet – the ideal constellation to bringing R&D efforts to business success.

### Financial Overview

<table>
<thead>
<tr>
<th>Financial overview</th>
<th>Full Year</th>
</tr>
</thead>
<tbody>
<tr>
<td>in CHF millions, except EPS (CHF) and number of shares (millions)</td>
<td>2021</td>
</tr>
<tr>
<td>Revenues</td>
<td>35</td>
</tr>
<tr>
<td>Operating expenses</td>
<td>(648)</td>
</tr>
<tr>
<td>Operating income (loss)</td>
<td>(613)</td>
</tr>
<tr>
<td>Net income (loss)</td>
<td>(635)</td>
</tr>
<tr>
<td>Basic EPS</td>
<td>(3.77)</td>
</tr>
<tr>
<td>Basic weighted average number of shares</td>
<td>168.5</td>
</tr>
<tr>
<td>Diluted EPS</td>
<td>(3.77)</td>
</tr>
<tr>
<td>Diluted weighted average number of shares</td>
<td>168.5</td>
</tr>
</tbody>
</table>

The full financial statements can be found in the Financial Report available on our corporate website.

* Idorsia measures, reports and issues guidance on non-GAAP operating performance. Idorsia believes that these non-GAAP financial measurements more accurately reflect the underlying business performance and therefore provide useful supplementary information to investors. These non-GAAP measures are reported in addition to, not as a substitute for, US GAAP financial performance.
Share Information

Idorsia was incorporated in March 2017 and listed on the SIX Swiss Exchange (ticker symbol: IDIA) in June 2017.

Idorsia Ltd is part of the following indices: SPI, SPIEX, SPI ESG, SXSLI, SXI Life Sciences, SXI Bio+Medtech, and SSIRT.

Idorsia is traded under the following symbols: Reuters IDIA.S / Bloomberg IDIA:SW.

Company Strategy

We will develop Idorsia into one of Europe’s leading biopharmaceutical companies, with a strong scientific core. We have identified five key strategic priorities to ensure the company’s success in the mid-term:

Deliver at least three products to market
We believe that our development compounds have the potential to significantly change treatment in their target diseases, resulting in medicines with substantial commercial potential.

Build a world-class commercial organization
In order to bring pioneering therapies to patients and to maximize the value of our innovations, we plan to continue building and integrating our global commercial organization.

Bring Idorsia to sustainable profitability
We are building Idorsia with a long-term focus and ambitious aspirations. By advancing our development pipeline and commercial readiness, we aim to bring Idorsia to sustainable profitability as soon as possible.

Fuel our pipeline with new discoveries
While building up our commercial operations and developing our late-stage clinical pipeline so as to bring innovative therapies to patients, we also continue to discover new compounds.

Utilize state-of-the-art technologies to drive innovation
As we wish to remain at the cutting edge of science, it is vital that we consider innovative approaches and utilize state-of-the-art technologies at each stage of the process, from bench to bedside.
# Clinical Development Pipeline

<table>
<thead>
<tr>
<th>Compound</th>
<th>Mechanism of Action</th>
<th>Target Indication</th>
<th>Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>Daridorexant</td>
<td>Dual orexin receptor antagonist</td>
<td>Insomnia</td>
<td>Approved as QUVIVIQ™ in the US, Under review in other countries</td>
</tr>
<tr>
<td>Aprocitentan*</td>
<td>Dual endothelin receptor antagonist</td>
<td>Resistant hypertension management</td>
<td>Phase 3 recruitment complete</td>
</tr>
<tr>
<td>Clazosentan</td>
<td>Endothelin receptor antagonist</td>
<td>Cerebral vasospasm associated with aneurysmal subarachnoid hemorrhage</td>
<td>Approved as PIVLAZ™ in Japan Global Phase 3</td>
</tr>
<tr>
<td>Lucerastat</td>
<td>Glucosylceramide synthase inhibitor</td>
<td>Fabry disease</td>
<td>Phase 3 – primary endpoint not met Open Label Extension (OLE) study ongoing</td>
</tr>
<tr>
<td>Selatogrel</td>
<td>P2Y12 receptor antagonist</td>
<td>Suspected acute myocardial infarction</td>
<td>Phase 3</td>
</tr>
<tr>
<td>Cenerimod</td>
<td>S1P1 receptor modulator</td>
<td>Systemic lupus erythematosus</td>
<td>Phase 3 in preparation</td>
</tr>
<tr>
<td>ACT-539313</td>
<td>Selective orexin 1 receptor antagonist</td>
<td>Binge eating disorder</td>
<td>Phase 2 recruitment complete</td>
</tr>
<tr>
<td>Sinbaglustat</td>
<td>GBA2/GCS inhibitor</td>
<td>Rare lysosomal storage disorders</td>
<td>Phase 1 complete</td>
</tr>
<tr>
<td>ACT-1004-1239</td>
<td>CXCR7 antagonist</td>
<td>Immunology</td>
<td>Phase 1 complete</td>
</tr>
<tr>
<td>ACT-1014-6470</td>
<td>-</td>
<td>Immunology</td>
<td>Phase 1</td>
</tr>
<tr>
<td>ACT-777991</td>
<td>-</td>
<td>Immunology</td>
<td>Phase 1</td>
</tr>
</tbody>
</table>

* 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 ACT-709478 (NBI-827104), Idorsia’s novel T-type calcium channel blocker. ACT-709478 is currently investigated in two Phase 2 studies for the treatment of a rare form of pediatric epilepsy and essential tremor.
Partnerships

**Johnson & Johnson**
In 2017, Idorsia and Actelion Pharmaceuticals, one of the Janssen Pharmaceutical Companies of Johnson & Johnson, entered into a revenue-sharing agreement in respect of ponesimod. In 2021, ponesimod was approved and subsequently launched in the US, Europe, and Canada to treat patients with relapsing forms of multiple sclerosis.
[www.investor.jnj.com](http://www.investor.jnj.com)

**Janssen Biotech**
In 2017, Idorsia entered into a collaboration agreement with Janssen Biotech, one of the Janssen Pharmaceutical Companies of Johnson & Johnson, to jointly develop aprocitentan and any of its derivative compounds or products. Janssen Biotech has sole commercialization rights worldwide. The results of the Phase 3 study of aprocitentan in patients with resistant hypertension are expected in mid-2022.
[www.janssen.com](http://www.janssen.com)

**Antares Pharma**
In 2019, Idorsia entered into a global agreement with Antares Pharma to develop a novel drug-device product combining selatogrel – Idorsia’s potent, fast-acting, reversible and highly selective P2Y12 receptor antagonist – with the Antares subcutaneous QuickShot® auto-injector. In 2021, Idorsia initiated the Phase 3 study SOS-AMI with the selatogrel drug-device for the treatment of suspected acute myocardial infarction.
[www.antarespharma.com](http://www.antarespharma.com)

**Mochida**
In 2019, Idorsia and Mochida Pharmaceutical entered into an exclusive license agreement for the supply, co-development and co-marketing of daridorexant, Idorsia’s dual orexin receptor antagonist, for insomnia and related disorders in Japan.
[www.mochida.co.jp](http://www.mochida.co.jp)

**Neurocrine**
In 2020, Idorsia entered into a global license agreement with Neurocrine Biosciences for the development and commercialization of ACT-709478, Idorsia’s potent, selective, orally active and brain-penetrating T-type calcium channel blocker, for the treatment of a rare form of pediatric epilepsy; in addition, a research collaboration was established to discover, identify and develop additional novel T-type calcium channel blockers. In 2021, Neurocrine also initiated a Phase 2 study in essential tremor.
[www.neurocrine.com](http://www.neurocrine.com)

**Santhera**
In 2020, Idorsia’s license, collaborative development and commercialization agreement with ReveraGen BioPharma in respect of vamorolone was transferred in its entirety to Santhera Pharmaceuticals, with the latter replacing Idorsia as a party to the agreement.
[www.santhera.com](http://www.santhera.com)

**Syneos Health**
In 2020, Idorsia and Syneos Health entered into an innovative commercial partnership to build the salesforce for the US launch of QUVIVIQ™ (daridorexant). In January 2022, Idorsia expanded this commercialization partnership to support the potential launch of daridorexant and effectively reach the primary care market in Europe and Canada.
[www.syneoshealth.com](http://www.syneoshealth.com)
Company milestones

2022

January
PIVLAZ™ (clazosentan) approved in Japan for the prevention of cerebral vasospasm, vasospasm-related cerebral infarction and cerebral ischemic symptoms after aSAH

January
The Lancet Neurology reports impact of daridorexant on both nighttime symptoms and daytime functioning in adults with insomnia

January
QUVIVIQ™ (daridorexant) 25 mg and 50 mg approved in the US for the treatment of adults with insomnia

2021

December
Idorsia to further characterize lucerastat for Fabry disease by continuing the open-label extension of the Phase 3 MODIFY study

November
Idorsia to advance cenerimod into Phase 3 development for SLE

September
Five Idorsia affiliates in key European markets (France, Germany, Italy, Spain, UK) established

2020

July
Establishment of Idorsia Pharmaceuticals US Inc. to perform commercial operations

February
Initiation of Phase 3 registration program with clazosentan for patients with cerebral vasospasm associated with aSAH

June
Initiation of Phase 3 registration study with selatogrel for the treatment of acute myocardial infarction

March – August
Marketing authorisation application (MAA) for daridorexant submitted to the EMA (March), Swissmedic (April), Health Canada (August)

2018

June
Initiation of Phase 3 registration study with aprocitentan for resistant hypertension management

June
Establishment of Idorsia Pharmaceuticals Japan to perform clinical development and commercial activities

2017

December
Collaboration Agreement with Janssen Biotech Inc. to jointly develop and commercialize aprocitentan

June
Idorsia opens its doors and is listed on SIX Swiss Stock Exchange

May
Global license agreement with Neurocrine Biosciences for the development and commercialization of ACT-709478

November
Collaboration Agreement with Antares for the development of a novel self-administered drug-device product for selatogrel
Idorsia is an independent biopharmaceutical company based on science and innovation. The company is specialized in the discovery and development of small molecules, to transform the horizon of therapeutic options. It is headquartered in Allschwil/Basel, Switzerland and is quoted on the SIX Swiss Exchange (tickersymbol: IDIA). All trademarks are legally protected by their respective owners.

Disclaimer This fact sheet has the sole purpose to provide members of the public with general information about the activities of Idorsia. The forward-looking statements in this fact sheet are based on current expectations and belief of company management, which are subject to numerous risks and uncertainties.

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