Media Release
July 26, 2022

Ad hoc announcement pursuant to Art. 53 LR

Idorsia announces financial results for the first half 2022 – reaching commercial stage

Allschwil, Switzerland – July 26, 2022
Idorsia Ltd (SIX: IDIA) today announced its financial results for the first half of 2022.

Commercial highlights

- **QUVIVIQ™ (daridorexant)** was launched in the US in May 2022 and is building positive early momentum in the insomnia market. Net sales in the first half of 2022 were CHF 0.4 million
- **PIVLAZ™ (clazosentan)** was launched in Japan in April 2022 with strong support from neurosurgeons treating aSAH patients. Net sales in the first half of 2022 were CHF 11.4 million

Pipeline highlights

- **QUVIVIQ (daridorexant)** – Europe's first dual orexin receptor antagonist – granted approval by the European Commission on April 29, 2022
- **Aprocitentan** – The Phase 3 study, PRECISION, demonstrated the antihypertensive effect of aprocitentan when added to standard of care in patients with resistant hypertension
- **Clazosentan** – Recruitment into the Phase 3 study, REACT, completed with 409 patients randomized
- **Cenerimod** – The analysis of the 12-month treatment data of the Phase 2b study has reinforced the decision to pursue cenerimod in a Phase 3 program – plans are being finalized

Financial highlights

- **Net revenue** HY 2022 at CHF 22 million
- **US GAAP operating expenses** HY 2022 at CHF 427 million
- **Non-GAAP operating expenses** HY 2022 at CHF 407 million
- **Guidance for 2022**: US GAAP operating loss of around CHF 840 million and non-GAAP operating loss of around CHF 785 million confirmed – unforeseen events excluded

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"I am very proud that within five years of Idorsia's founding, we have received approvals for two of our products, built a commercial organization from scratch, and launched in two of the world's largest pharmaceutical markets. At the same time, we are ensuring the future growth of the company by continuing to advance our clinical development pipeline, as evidenced this past quarter with the successful conclusion of the Phase 3 clinical study for aprocitentan, putting us on track for our third NDA. Our transformation to a fully-fledged biopharmaceutical company, including commercial capabilities, is happening now!"

Financial results

US GAAP results in CHF millions, except EPS (CHF) and number of shares (millions)	First Half		Second Quarter	
	2022	2021	2022	2021
Net revenues	22	14	17	7
Operating expenses	(427)	(265)	(229)	(137)
Operating income (loss)	(405)	(252)	(212)	(130)
Net income (loss)	(419)	(243)	(222)	(139)
Basic EPS	(2.36)	(1.46)	(1.25)	(0.83)
Basic weighted average number of shares	177.3	166.9	177.5	167.1
Diluted EPS	(2.36)	(1.46)	(1.25)	(0.83)
Diluted weighted average number of shares	177.3	166.9	177.5	167.1

US GAAP net revenue of CHF 22 million in the first half of 2022 consisted of product sales of QUVIVIQ (CHF 0.4 million) and PIVLAZ (CHF 11.4 million), contract revenue recognized in connection with the collaboration agreements with Janssen Biotech, Inc. (CHF 5 million), Mochida Pharmaceutical Co., Ltd (CHF 3 million) and Neurocrine Biosciences, Inc. (CHF 2 million) and revenue share from J&J (CHF 0.6 million), compared to a revenue of CHF 14 million in the first half of 2021.

US GAAP operating expenses in the first half of 2022 amounted to CHF 427 million (CHF 265 million in the first half of 2021), of which CHF 1 million relates to cost of sales, CHF 192 million relates to R&D (CHF 192 million in the first half of 2021) and CHF 234 million to SG&A expenses (CHF 74 million in the first half of 2021).

US GAAP net loss in the first half of 2022 amounted to CHF 419 million compared to CHF 243 million in the first half of 2021. The increase of the net loss was mainly driven by higher operating expenses, mainly in the commercial functions and a negative financial result, partially offset by higher net sales.

The US GAAP net loss resulted in a net loss per share of CHF 2.36 (basic and diluted) in the first half of 2022 compared to a net loss per share of CHF 1.46 (basic and diluted) in the first half of 2021.

Non-GAAP* measures in CHF millions, except EPS (CHF) and number of shares (millions)	First Half		Second Quarter	
	2022	2021	2022	2021
Net revenues	22	14	17	7
Operating expenses	(407)	(248)	(219)	(128)
Operating income (loss)	(384)	(234)	(202)	(121)
Net income (loss)	(395)	(223)	(206)	(128)
Basic EPS	(2.23)	(1.34)	(1.16)	(0.77)
Basic weighted average number of shares	177.3	166.9	177.5	167.1
Diluted EPS	(2.23)	(1.34)	(1.16)	(0.77)
Diluted weighted average number of shares	177.3	166.9	177.5	167.1

** 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.*

Non-GAAP net loss in the first half of 2022 amounted to CHF 395 million: the CHF 23 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 9 million), share-based compensation (CHF 11 million) and a negative non-cash financial result (CHF 4 million).

The non-GAAP net loss resulted in a net loss per share of CHF 2.23 (basic and diluted) in the first half of 2022 compared to a net loss per share of CHF 1.34 (basic and diluted) in the first half of 2021.

Financial outlook

On April 20, 2022, the company launched PIVLAZ (clazosentan) in Japan. The company also launched QUVIVIQ (daridorexant) in the US on May 2, 2022. On April 29, 2022, QUVIVIQ (daridorexant) was approved in the European Union and is expected to be launched in the first European market before the end of the year. The clinical pipeline advanced substantially with highlights including the positive results of the Phase 3 registration study for aprocitentan, the ramp-up of recruitment into the pivotal SOS-AMI study with selatogrel, and the planned launch of the Phase 3 study of cenerimod. Accounting for all these activities, the company continues to anticipate a US GAAP operating loss of around CHF 840 million and a non-GAAP operating loss of around CHF 785 million for 2022, unforeseen events excluded.

The company is committed to become profitable and with the current forecasts continues to expect to reach this goal in 2025 with annual net sales above CHF 1 billion.

André C. Muller, Chief Financial Officer, commented:
"Idorsia has reached another important milestone by recording its first product sales with the launches of PIVLAZ in Japan and QUVIVIQ in the US. Becoming a commercial stage company is a key milestone to achieve our objective of reaching sustainable profitability by 2025. The positive readout of aprocitentan in June is another key milestone as the royalty entitlement from Janssen will bring us another source of revenue by 2024. All these recent milestones make us confident that we will be able to raise cash with non-equity dilutive instruments in the second half of 2022. The next few months will give us more visibility on the uptake of QUVIVIQ in the US. Irrespective of the net sales numbers that will be achieved for the full year 2022, we are fully committed to delivering our current guidance with a non-GAAP operating loss around CHF 785 million and a US GAAP operating loss around CHF 840 million for the full year 2022."

Liquidity and indebtedness

At the end of the first half of 2022, Idorsia's liquidity (including cash, cash equivalents and short-term deposits) amounted to CHF 733 million.

(in CHF millions)	Jun 30, 2022	Mar 31, 2022	Dec 31, 2021
Liquidity			
Cash and cash equivalents	233	146	101
Short-term deposits	500	794	927
Long-term deposits	-	-	160
Total liquidity*	**733**	**940**	**1,188**
Indebtedness			
Convertible loan	335	335	298
Convertible bond	795	794	794
Other financial debt	-	-	-
Total indebtedness	**1,129**	**1,129**	**1,093**

*rounding differences may occur

Commercial operations

In Q2 2022, Idorsia entered a new stage of the company's growth with the launches of QUVIVIQ in the US and PIVLAZ in Japan, as well as the approval of QUVIVIQ in the European Union.

Simon Jose, Chief Commercial Officer of Idorsia, commented:
"The feedback we are hearing from physicians and patients with insomnia in the US since QUVIVIQ launched in May has been very positive. The number of prescribers and dispensed prescriptions are growing steadily week over week, with the majority of prescribers adopting the 50mg dose and writing refills. As we start to ramp-up our branded direct-to-consumer campaigns and exit the summer months, I am confident that this positive momentum will continue to build. I am also looking forward to launching in Europe later this year, where QUVIVIQ will be the first dual orexin receptor antagonist, representing a significant medical advancement for the millions of patients who suffer from insomnia. In Japan, we are hearing a great deal of excitement about PIVLAZ from neurosurgeons treating aSAH patients. Approximately 60% of target accounts have ordered PIVLAZ as it continues to gain inclusion in hospital-level formularies and treatment protocols."

United States

Product	Mechanism of Action	Indication	Commercially available since
QUVIVIQ (daridorexant)	Dual orexin receptor antagonist	Treatment of adult patients with insomnia, characterized by difficulties with sleep onset and/or sleep maintenance	2 May 2022

QUVIVIQ (daridorexant) was launched in the US in May, offering a new treatment option to help patients fall asleep faster and stay asleep longer, to the millions of American adults suffering from insomnia. Early feedback from healthcare professionals (HCPs) and patients has been very positive.

Since the launch in May, sales have reached CHF 0.4 million. To enable early patient access we are providing a robust patient support and copay program, including a free first 30-day prescription; hence the net sales number does not represent actual prescriptions or product demand.

The number of prescribers, as well as prescriptions dispensed, continues to grow week over week. Notably, approximately 60 percent of prescriptions were written with one or more refills and approximately 75 percent were written for the 50 mg strength, the dose expected to offer the best efficacy for the majority of patients. These are encouraging signs that our highly trained US Sales Team is executing the customer interaction strategy and successfully delivering our evidence-based messages to targeted HCPs.

To help further this positive momentum, we are in the process of launching several branded direct-to-consumer (DTC) initiatives, including partnering with recently announced QUVIVIQ patient ambassadors, actor Taye Diggs and former skiing world champion Lindsey Vonn, both of whom are being prescribed QUVIVIQ to treat their insomnia, and having positive experiences with the product. Taye and Lindsey recently spoke with a number of media outlets, including *Good Day New York*, the *TODAY show, People magazine* and *Essence*, to discuss their struggles with insomnia and the impact taking QUVIVIQ nightly is having on them. DTC advertising featuring both Taye and Lindsey is

scheduled to begin in late summer across the full spectrum of broadcast, digital and print platforms. We are also continuing our unbranded educational campaign, *Seize the Night & Day*, featuring Jennifer Aniston, to drive broad awareness about insomnia.

For more information about QUVIVIQ in the US, see the Full Prescribing Information (PI and Medication Guide).

Japan

Product	Mechanism of Action	Indication	Commercially available since
PIVLAZ (clazosentan) PIVLAZ clazosentan	Endothelin receptor antagonist	Prevention of cerebral vasospasm, vasospasm-related cerebral infarction and cerebral ischemic symptoms after aneurysmal subarachnoid hemorrhage (aSAH)	20 April 2022

PIVLAZ (clazosentan) was launched in Japan and made available to patients suffering from aneurysmal subarachnoid hemorrhage (aSAH) in April 2022. The Idorsia Japan team is actively engaging with the medical community and neurosurgeons to raise awareness about this life-threatening condition and share the PIVLAZ clinical data.

As the first innovative treatment for aSAH in 25 years in Japan, neurosurgeons recognize the clinical safety and efficacy of PIVLAZ and the importance of preventing cerebral vasospasm and vasospasm-related cerebral infarction and cerebral ischemic symptoms in aSAH patients. PIVLAZ is already being incorporated into hospital formularies and treatment protocols and approximately 60% of target accounts have placed at least one order for PIVLAZ. Based on the estimated incidence of aSAH in Japan, approximately 10% of aSAH patients were treated with PIVLAZ in June 2022. Since the launch in April, net sales have reached CHF 11.4 million.

Europe and Canada region
QUVIVIQ (daridorexant) was approved by the European Commission in April 2022 for the treatment of adult patients with insomnia characterized by symptoms present for at least three months and considerable impact on daytime functioning. For more information about QUVIVIQ in the EU, see the Summary of Product Characteristics.

Launch preparations are underway in the top five European markets with the first launch in Germany expected before the end of the year. The local teams are actively engaging with medical experts, policy makers and payors to raise awareness about the significant burden of chronic insomnia disorder and introduce QUVIVIQ. As the first dual orexin receptor antagonist approved in Europe, there is a high level of interest among medical experts in the mechanism of action and clinical results for QUVIVIQ.

Clinical Development

Idorsia's diversified and balanced clinical development pipeline, which covers multiple therapeutic areas, including CNS, cardiovascular and immunological disorders, as well as orphan diseases, has made significant progress in the second quarter of 2022.

Daridorexant was approved by the US FDA in January 2022 and by the European Commission in April 2022. In the UK, the formal approval by the Medicines and Healthcare products Regulatory Agency (MHRA) is expected soon. Daridorexant is currently under review with Swissmedic and Health Canada. In Japan, a Phase 3 study with daridorexant has completed recruitment. Idorsia is committed to further evaluating the safety and efficacy of daridorexant in populations of high medical need, including pediatrics, where there is the need for a pharmacological treatment with a favorable benefit/risk profile supported by adequate, controlled clinical studies. Therefore, Idorsia has initiated a Phase 2, double-blind, randomized, placebo-controlled, dose-finding study to assess the efficacy, safety, and pharmacokinetics of multiple-dose oral administration of daridorexant in pediatric patients aged between 10 and < 18 years with insomnia disorder. The primary objective of the study is to characterize the dose-response relationship of daridorexant on total sleep time (TST) using polysomnography. The study is expected to enroll around 150 patients, who will be randomized in a 1:1:1:1 ratio to 10, 25, 50 mg daridorexant, or placebo. The development program has been designed based on advice and agreement with the US FDA and the EU PDCO via a Pediatric Study Plan and Pediatric Investigational Plan.

In May 2022, Idorsia announced positive top-line results of **PRECISION**, the Phase 3 study investigating **aprocitentan** for the treatment of patients whose blood pressure is not adequately controlled despite receiving at least triple antihypertensive therapy. Aprocitentan significantly reduced blood pressure when added to standardized combination background antihypertensive therapy in patients with resistant hypertension over 48 weeks of treatment. The company will now discuss the results with health authorities with the aim to file the new drug application for aprocitentan with the US FDA by the end of the year, closely followed by other health authorities. Idorsia will also make the detailed results of the Phase 3 study available through scientific presentations and peer-reviewed publications. More information can be found in the dedicated press release.

REACT, the global Phase 3 study with **clazosentan** for the prevention of clinical deterioration due to vasospasm-related delayed cerebral ischemia following aneurysmal subarachnoid hemorrhage, has completed recruitment with 409 patients – treated either with microsurgical clipping or endovascular coiling – randomized 1:1 to placebo or clazosentan 15 mg/h. The study is expected to conclude by the end of 2022, reporting results in the first quarter of 2023.

In December 2021, Idorsia reported that after the planned interim analysis of the open-label extension (OLE) of the Phase 3 **MODIFY** study with **lucerastat** for the treatment of adult patients with Fabry disease, the study will continue. The company will consult with health authorities in the first half of 2023 and discuss the additional data collected in the OLE study. The data includes the placebo-controlled 6-month treatment period with 118 patients in MODIFY, as well as the analysis of 107 patients who continued into the OLE, many of whom are treated with lucerastat for one year and some whom have received treatment for up to 2 years.

The Phase 3 study with **selatogrel** is currently recruiting patients, with a target enrollment of approximately 14,000 patients who are at high risk of recurrent acute myocardial infarction. The recruitment of patients is ramping up as more sites are initiated, with a target of more than 500 sites in about 45 countries.

The Phase 2b study with **cenerimod** has concluded the 12-month treatment period and is in the safety follow up period. The analysis of the 12-month treatment data has reinforced the decision to pursue cenerimod for the treatment of systemic lupus erythematosus in a Phase 3 program. Following discussions with health authorities, the plans for the Phase 3 program are being finalized.

ACT-539313 is a potent, brain-penetrating, selective orexin 1 receptor antagonist. In the Phase 1 studies, ACT-539313 was well tolerated at single oral doses of up to and including 400 mg and at multiple oral doses of up to and including 200 mg twice daily for 10 days in healthy volunteers. Following an unsuccessful proof-of-concept study in binge eating disorder, the company is fully analyzing the data and expects to publish the results of the study in scientific literature.

ACT-1004-1239 is a first-in-class, potent, selective ACKR3/CXCR7 antagonist. Preclinical data has shown both anti-inflammatory and promyelinating effects. The Phase 1 program is complete, and following feedback from the US FDA, we are finalizing the plan for a Phase 2 study with ACT-1004-1239 in multiple sclerosis.

Idorsia's clinical development pipeline

Compound	Mechanism of Action	Target Indication	Status
Daridorexant	Dual orexin receptor antagonist	Insomnia	Approved in the EU Under review in Switzerland and Canada Phase 3 in Japan – recruitment complete Phase 2 in pediatric insomnia – recruiting
Aprocitentan*	Dual endothelin receptor antagonist	Resistant hypertension management	Phase 3 successful – filing by end 2022
Clazosentan	Endothelin receptor antagonist	Cerebral vasospasm assoc. with aneurysmal subarachnoid hemorrhage	Global Phase 3 – recruitment complete
Lucerastat	Glucosylceramide synthase inhibitor	Fabry disease	Phase 3 primary endpoint not met, OLE ongoing**
Selatogrel	$P2Y_{12}$ receptor antagonist	Suspected acute myocardial infarction	Phase 3 recruiting
Cenerimod	$S1P_1$ receptor modulator	Systemic lupus erythematosus	Phase 3 in preparation
ACT-539313	Selective orexin 1 receptor antagonist	Under evaluation	-
ACT-1004-1239	ACKR3 / CXCR7 antagonist	Multiple Sclerosis	Phase 2 in preparation
Sinbaglustat	GBA2/GCS inhibitor	Rare lysosomal storage disorders	Phase 1 complete
ACT-1014-6470	-	Immunology	Phase 1
ACT-777991	-	Immunology	Phase 1

** In collaboration with Janssen Biotech to jointly develop aprocitentan, Janssen Biotech has sole commercialization rights worldwide*
*** Open-label extension study*

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.

Further details including the current status of each project in our portfolio can be found in our innovation fact sheet.

Half-year financial report

A full financial update is available in Idorsia's 2022 Half Year Financial Report, at www.idorsia.com/investors/corporate-reports.

Results Day Center

Investor community: To make your job easier, we provide all relevant documentation via the Results Day Center on our corporate website: www.idorsia.com/results-day-center.

Upcoming Financial Updates

- Nine-months 2022 Financial Results reporting on October 25, 2022
- Full-Year 2022 Financial Results reporting on February 7, 2023
- First Quarter 2023 Financial Results reporting on April 25, 2023

Notes to the editor

About Idorsia

Idorsia Ltd is reaching out for more – We have more ideas, we see more opportunities and we want to help more patients. In order to achieve this, we will develop Idorsia into a leading biopharmaceutical company, with a strong scientific core.

Headquartered near Basel, Switzerland – a European biotech-hub – Idorsia is specialized in the discovery, development and commercialization of small molecules to transform the horizon of therapeutic options. Idorsia has a broad portfolio of innovative drugs in the pipeline, an experienced team of professionals covering all disciplines from bench to bedside, state-of-the-art facilities, and a strong balance sheet – the ideal constellation to translate R&D efforts into business success.

Idorsia was listed on the SIX Swiss Exchange (ticker symbol: IDIA) in June 2017 and has over 1,200 highly qualified specialists dedicated to realizing our ambitious targets.

For further information, please contact

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