Media Release
October 25, 2022

Ad hoc announcement pursuant to Art. 53 LR

Idorsia announces financial results for the first nine months of 2022 – QUVIVIQ becoming a global brand

Allschwil, Switzerland – October 25, 2022
Idorsia Ltd (SIX: IDIA) today announced its financial results for the first nine months of 2022.

Commercial highlights

- **QUVIVIQ™ (daridorexant)** acceleration in volume and number of patients treated after the start of the consumer campaign – poised to become the leading branded insomnia medication in the US in new-to-brand prescriptions
- **QUVIVIQ™ (daridorexant)** Net sales of CHF 2.3 million since launch in May 2022, do not reflect the volume as broad commercial coverage is under negotiation
- **PIVLAZ™ (clazosentan)** strong performance in Japan with net sales of CHF 25.1 million since launch in April 2022, with approximately 20% of aSAH patients treated in September

Pipeline highlights

- **QUVIVIQ (daridorexant)** – Europe's first DORA – granted approval by the European Commission in April 2022 and subsequently by the Medicines and Healthcare products Regulatory Agency in Great Britain, approval in Switzerland anticipated in the coming months
- **Daridorexant** – Positive Phase 3 study of Japanese patients with insomnia paves way to regulatory submission in H1 2023
- **Aprocitentan** – Data from the positive Phase 3 study, PRECISION, will be presented as a Late-Breaking Science presentation during the American Heart Association (AHA) Scientific Sessions 2022. Activities to submit a New Drug Application to the US FDA are on track for filing by the end of the year.
- **Clazosentan** – Market registration, based on Japanese data, requested in Republic of Korea
- **Clazosentan** – Results from global Phase 3 study, REACT, expected in the first quarter 2023
- **Cenerimod** – Data from the Phase 2b study, CARE, will be presented at the American College of Rheumatology (ACR) Convergence 2022

Financial highlights

- **Net revenue** 9M 2022 at CHF 43 million
- **US GAAP operating expenses** 9M 2022 at CHF 653 million
- **Non-GAAP operating expenses** 9M 2022 at CHF 621 million
- **Sale & leaseback** – Idorsia entered into an agreement generating proceeds of CHF 164 million
- **Guidance for 2022**: US GAAP operating loss of around CHF 840 million and non-GAAP operating loss of around CHF 785 million confirmed – barring unforeseen events

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"Idorsia is making great progress with the transformation into a commercial company. QUVIVIQ will soon be the leading branded insomnia medication in the US in terms of new prescriptions. The increased volume following the direct-to-consumer campaign supports the ongoing negotiations to secure reimbursement. We are just weeks away from a first-in-class product launch in Europe, and the positive data in Japan means that we are well on track to making daridorexant a global product for patients with insomnia. With such a long patent life ahead, QUVIVIQ will deliver long-term growth and support the advancement of our other innovations. In parallel, PIVLAZ is off to a strong start in Japan with around 20% of all aSAH patients receiving PIVLAZ in the last month. This is all being achieved while our R&D continues to deliver, and I look forward to seeing the results of some key studies presented at upcoming congresses in the next few weeks."

Financial results

US GAAP results in CHF millions, except EPS (CHF) and number of shares (millions)	Nine Months		Third Quarter	
	2022	2021	2022	2021
Net revenues	43	30	21	17
Operating expenses	(653)	(415)	(227)	(150)
Operating income (loss)	(610)	(385)	(206)	(133)
Net income (loss)	(635)	(383)	(216)	(140)
Basic EPS	(3.58)	(2.29)	(1.22)	(0.83)
Basic weighted average number of shares	177.4	167.0	177.5	167.3
Diluted EPS	(3.58)	(2.29)	(1.22)	(0.83)
Diluted weighted average number of shares	177.4	167.0	177.5	167.3

US GAAP net revenue of CHF 43 million in the first nine months of 2022 (CHF 30 million in the first nine months of 2021) consisted of product sales of QUVIVIQ (CHF 2.3 million) and PIVLAZ (CHF 25.1 million), contract revenue recognized in connection with the collaboration agreements with Janssen Biotech, Inc. (CHF 8 million), Mochida Pharmaceutical Co., Ltd (CHF 4 million) and Neurocrine Biosciences, Inc. (CHF 3 million), and revenue share from J&J (CHF 1.3 million).

US GAAP operating expenses in the first nine months of 2022 amounted to CHF 653 million (CHF 415 million in the first nine months of 2021), of which CHF 4 million relates to cost of sales, CHF 278 million to R&D expenses (CHF 288 million in the first nine months of 2021) and CHF 372 million to SG&A expenses (CHF 127 million in the first nine months of 2021).

US GAAP net loss in the first nine months of 2022 amounted to CHF 635 million (CHF 383 million in the first nine months of 2021). The increase of the net loss was mainly driven by higher operating expenses, largely in the commercial functions, and a negative financial result, partially offset by higher net revenues.

The US GAAP net loss resulted in a net loss per share of CHF 3.58 (basic and diluted) in the first nine months of 2022, compared to a net loss per share of CHF 2.29 (basic and diluted) in the first nine months of 2021.

Non-GAAP* measures	Nine Months		Third Quarter	
in CHF millions, except EPS (CHF) and number of shares (millions)	2022	2021	2022	2021
Net revenues	43	30	21	17
Operating expenses	(621)	(388)	(214)	(139)
Operating income (loss)	(577)	(357)	(193)	(123)
Net income (loss)	(597)	(347)	(202)	(124)
Basic EPS	(3.36)	(2.08)	(1.14)	(0.74)
Basic weighted average number of shares	177.4	167.0	177.5	167.3
Diluted EPS	(3.36)	(2.08)	(1.14)	(0.74)
Diluted weighted average number of shares	177.4	167.0	177.5	167.3

** 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 nine months of 2022 amounted to CHF 597 million: the CHF 38 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 13 million), share-based compensation (CHF 19 million) and a negative non-cash financial result (CHF 6 million).

The non-GAAP net loss resulted in a net loss per share of CHF 3.36 (basic and diluted) in the first nine months of 2022, compared to a net loss per share of CHF 2.08 (basic and diluted) in the first nine months of 2021.

Sale and leaseback

In September 2022, Idorsia entered into a sale and leaseback agreement with a private Swiss company for Idorsia's research and development building at its headquarters in Allschwil. The building was sold for CHF 164 million (net proceeds of CHF 162 million) and leased back by Idorsia from October 1, 2022.

Financial outlook

In the second quarter of 2022, Idorsia launched two products in two of the world's largest pharmaceutical markets: PIVLAZ (clazosentan) in Japan on April 20 and QUVIVIQ (daridorexant) in the US on May 2. On April 29, 2022, QUVIVIQ (daridorexant) was approved in the European Union and subsequently in Great Britain by Medicines and Healthcare products Regulatory Agency, while approval in Switzerland is expected in the coming months. As a result, QUVIVIQ will 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 and the Japanese Phase 3 study of daridorexant, the completion of recruitment into the Phase 3 study of clazosentan, the ramp-up of recruitment into the pivotal SOS-AMI study with selatogrel, and the planned initiation 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, barring unforeseen events.

The company is committed to becoming profitable and continues to expect to reach this goal in 2025, with annual net sales above CHF 1 billion.

André C. Muller, Chief Financial Officer, commented:
"While the company continues to execute on the strategic priorities, I am maintaining my focus on ensuring financing to bridge the gap until reaching profitability. I remain confident that we will secure additional funding in 2022 with a non-equity dilutive instrument. Once again, I reiterate that we are fully committed to delivering on our current guidance despite the unfavorable currency environment."

Liquidity and indebtedness

At the end of the first nine months of 2022, Idorsia's liquidity (including cash, cash equivalents and short-term deposits) amounted to CHF 695 million.

(in CHF millions)	Sep 30, 2022	Jun 30, 2022	Dec 31, 2021
Liquidity			
Cash and cash equivalents	315	233	101
Short-term deposits	380	500	927
Long-term deposits	-	-	160
Total liquidity*	**695**	**733**	**1,188**
Indebtedness			
Convertible loan	335	335	298
Convertible bond	795	795	794
Other financial debt	162	-	-
Total indebtedness	**1,291**	**1,129**	**1,093**

*rounding differences may occur

Commercial operations

In the third quarter of 2022, Idorsia continued to gain momentum with the rollout of QUVIVIQ™ (daridorexant) in the US and PIVLAZ™ (clazosentan) in Japan, while preparing for the launch of QUVIVIQ in Europe.

Simon Jose, Chief Commercial Officer of Idorsia, commented:
"I'm determined that patients will get to benefit from our innovation by making QUVIVIQ a leading insomnia medication on a global basis. The growth in demand of QUVIVIQ in the US means that we are close to becoming the leading branded insomnia medication in new-to-brand prescriptions, only 6 months after launch. While I'm pleased this milestone is imminent, this is just the beginning for QUVIVIQ. Our marketing efforts, including direct-to-consumer advertising, are generating new patient starts and we are seeing an increasing number of refills for patients who have started QUVIVIQ. The increase in demand provides a good basis to support our negotiations with payors. We are also just weeks away from the launch of QUVIVIQ in Germany and Italy, which will mark the availability of the first and only DORA in Europe. In the meantime, PIVLAZ is performing strongly in Japan. We are seeing continued month-over-month growth in patients treated and the number of hospitals including PIVLAZ in their 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	May 2, 2022

QUVIVIQ (daridorexant) has been on the market in the US for just over five months and continues to gain market share. QUVIVIQ is poised to become the leading branded insomnia medication in new-to-brand prescriptions (NBRx). Not only is there a steady flow of new-to-brand patients, but encouragingly, refills are increasing on a weekly basis – evidence suggestive of patient satisfaction with the efficacy of the product.

Since the launch in May, net sales have reached CHF 2.3 million. As highlighted at half-year, to enable early patient access to QUVIVIQ, the company continues to offer a strong copay program, including a free first 30-day prescription. This strategy is helping to support early product uptake, which is an important proof point in discussions with payers. However, due to this approach, the net sales numbers do not reflect actual dispensed prescriptions or product demand.

Since product launch, QUVIVIQ has shown strong month-over-month growth in prescriptions, with a total of over 10,000 prescriptions dispensed in September. The number of both new and repeat prescribers also continues to grow with a similar trajectory. In addition, more than 70 percent of prescriptions are for 50 mg, the dose strength expected to offer the best outcome for the majority of patients.

In August, the first direct-to-consumer (DTC) national television commercial was launched in the US, featuring former skiing world champion Lindsey Vonn. The second commercial, featuring actor Taye Diggs, aired just a few weeks ago, and both commercials are currently running on US network television and streaming channels. Both Lindsey and Taye are patient ambassadors currently taking QUVIVIQ and having positive experiences with the product. The commercials, as well as "surround sound" messaging via organic, paid and social media, resulted in an immediate increase in patient requests for QUVIVIQ and as a consequence, an increase in new-to-brand prescriptions.

Coupled with the ongoing unbranded educational *Seize the Night & Day* campaign, featuring Jennifer Aniston and newly announced partner, former Olympic sprinter Gabby Thomas, these campaigns are expected to continue to drive patient awareness and prescriptions.

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

Europe and Canada region
In April 2022, marketing authorization for QUVIVIQ was granted by the European Commission and subsequently by the Medicines and Healthcare products Regulatory Agency (MHRA) in Great Britain via the European Commission Decision Reliance Procedure. For more information about QUVIVIQ in the EU, see the Summary of Product Characteristics.

The launches of QUVIVIQ in Germany and Italy are planned for November 2022, making QUVIVIQ the first dual orexin receptor antagonist available to the millions of patients suffering from chronic insomnia in Europe. The launch preparations in the major European markets are well on track, and there is growing excitement about the differentiated profile of QUVIVIQ among medical experts. The local teams are actively engaging with reimbursement authorities to provide patients with access to QUVIVIQ.

Daridorexant is currently under review with Swissmedic and Health Canada.

Japan

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

PIVLAZ (clazosentan) was launched in Japan in April 2022 for the prevention of cerebral vasospasm, vasospasm-related cerebral infarction and cerebral ischemic symptoms in patients suffering from aneurysmal subarachnoid hemorrhage (aSAH). With an innovative mechanism of action and proven efficacy and safety in Japanese patients, PIVLAZ is gaining inclusion in hospital formularies and aSAH treatment protocols. The number of new accounts opened continues to increase, with over 80% of target accounts having ordered PIVLAZ. Based on the estimated incidence of aSAH in Japan, approximately 20% of aSAH patients were treated with PIVLAZ in September 2022. Since the launch in April, net sales have reached CHF 25.1 million.

Idorsia has established an affiliate in the Republic of Korea in preparation for the potential launch of clazosentan, following the filing of a request for market registration based on data from the Japanese clinical program.

Clinical development

Idorsia's diversified and balanced clinical development pipeline – covering multiple therapeutic areas, including CNS, cardiovascular and immunological disorders, as well as orphan diseases – has made significant progress in the first nine months of 2022.

Daridorexant is currently under review with Swissmedic and Health Canada. In Japan, a Phase 3 study with daridorexant reported positive top-line results in October 2022, paving the way for the filing of a New Drug Application with the Japanese Ministry of Health, Labor and Welfare in the first half of 2023. More information can be found in the dedicated press release. Idorsia has initiated a Phase 2 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 development program has been designed in consultation with the US FDA and the EU PDCO on the basis of a Pediatric Study Plan and a Pediatric Investigation 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. More information can be found in the dedicated press release. The company is now discussing the results with health authorities, with the aim of filing the New Drug Application for aprocitentan with the US FDA by the end of the year, closely followed by other health authorities. Idorsia is also making the detailed results of the Phase 3 study available through scientific presentations and peer-reviewed publications, beginning with the Late-Breaking Science presentation scheduled for Monday, November 7 (15:10 – 15:20) at the American Heart Association (AHA) Scientific Sessions 2022, entitled “Sustained blood

pressure lowering effect with the dual endothelin receptor antagonist aprocitentan in resistant hypertension: results from a randomized, controlled study including a withdrawal phase".

A request for market registration for **clazosentan**, based on the data from the Japanese clinical program, has been filed with the Ministry of Health and Welfare in the Republic of Korea. A decision is expected in the second half of 2023. **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/hour. The study is expected to conclude by the end of 2022, with results to be reported in the first quarter of 2023.

In October 2022, Idorsia conducted another 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 data includes the placebo-controlled 6-month treatment period, as well as the OLE study patients, all of whom have now been treated with lucerastat for at least 12 months. The analysis confirmed the long-term effect on the reduction of plasma Gb3 and showed that the signal seen on kidney function after 6 months of treatment is confirmed after the longer treatment duration, supporting a potential long-term effect on kidney function. The analysis also confirmed a safety and tolerability profile consistent with that observed during the 6-month randomized treatment period. The OLE study will continue, and the company intends to consult with health authorities in the first half of 2023 to discuss the additional data collected.

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

The Phase 2b CARE study with **cenerimod** has concluded the 12-month treatment period, and the analysis of the data has reinforced the decision to pursue cenerimod 4 mg for the treatment of systemic lupus erythematosus in a Phase 3 program. The program has been discussed with health authorities, and activities to initiate the studies are underway. Data from CARE are being presented in an oral presentation by Anca Askanase, MD, MPH, (Founder and Clinical Director of the Columbia Lupus Center, and Associate Professor of Medicine, Columbia University) and in a poster presentation by Daniel Strasser (Senior Scientific Expert Clinical Biomarkers, Idorsia) at the American College of Rheumatology (ACR) Convergence 2022, which will be held on November 10–14, 2022.
Abstract number 1656; Efficacy and Safety of Cenerimod in Patients with Moderate to Severe Systemic Lupus Erythematosus (SLE): A Multicenter, Randomized, Parallel-Group, Double-Blind, Placebo-Controlled, Dose-Finding Phase 2b Trial.
Abstract number 1002: Investigation of Pharmacodynamic Biomarkers in a Phase 2b Study in Patients with Moderate to Severe SLE Treated with the $S1P_1$ Receptor Modulator Cenerimod.

Alberto Gimona, MD and Head of Global Clinical Development, Idorsia, commented:
"We are very pleased that the CARE study will be presented at the ACR meeting, and I'm looking forward to the lupus community seeing that cenerimod 4 mg consistently showed clinically meaningful and sustained improvement on multiple measures of SLE disease activity compared to placebo from baseline. This consistent effect of the 4 mg dose was particularly evident in patients with markers associated with more severe disease. We also saw a good safety profile, consistent with the mechanism of action, allowing us to move forward with the dose offering optimal efficacy. In summary, CARE was extremely helpful in informing the design of the Phase 3 program. The team is now initiating the Phase 3 program."

Idorsia's clinical development pipeline

Compound	Mechanism of action	Target indication	Status
Daridorexant	Dual orexin receptor antagonist	Insomnia	Approved in the EU & UK Under review in Switzerland and Canada Phase 3 in Japan successful – filing expected in H1 2023 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	CXCR3 antagonist	Recent-onset Type 1 diabetes	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 being investigated in a Phase 2 study for the treatment of a rare form of pediatric epilepsy.

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

Nine-month financial report

A full financial update is available in Idorsia's Financial Report Nine-Month 2022, at https://www.idorsia.com/investors/financial-information/financial-archive.

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

- Full-Year 2022 Financial Results reporting on February 7, 2023
- First Quarter 2023 Financial Results reporting on April 25, 2023
- Annual General Meeting of Shareholders on May 4, 2023
- Half-Year 2023 Financial Results reporting on July 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.

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The above 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.