Media Release
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Ad hoc announcement pursuant to Art. 53 LR

Idorsia announces financial results for the first quarter 2024 – advancing the company with renewed vigor

Allschwil, Switzerland – May 21, 2024
Idorsia Ltd (SIX: IDIA) today announced its financial results for the first quarter of 2024.

Business highlights

- **Viatris collaboration**: Global research and development collaboration, focused on the development and commercialization of two innovative compounds, selatogrel and cenerimod.

Commercial highlights

- **QUVIVIQ™ (daridorexant):** Total net sales of CHF 10 million in Q1 2024.
- **QUVIVIQ in the US:** Citizens petition to deschedule the DORA class progressing.
- **QUVIVIQ in Europe:** Further launches, including France, provide a solid base to increase European sales in 2024.

Pipeline highlights

- **TRYVIO™ (aprocitentan):** Approved by the US FDA in March 2024.
- **JERAYGO™ (aprocitentan):** Recommended for approval in Europe.
- **Daridorexant:** Phase 3 study conducted by Simcere in Chinese patients fully recruited.

Financial highlights

- **Net revenue** Q1 2024 at CHF 10 million.
- **US GAAP operating expenses** Q1 2024 benefiting from extraordinary income from Viatris deal with **non-GAAP operating expenses** Q1 2024 at CHF 96 million.
- **US GAAP operating income** Q1 2024 of CHF 31 million and **non-GAAP operating loss** of CHF 85 million.
- **The Viatris deal:** The upfront consideration of USD 350 million (CHF 308 million) was fully paid by Viatris to Idorsia in Q1 2024.
- **Convertible bond 2024:** Bondholders approve an extension of maturity by six months.

Guidance for 2024

- **QUVIVIQ net sales** of around CHF 55 million.
- **US GAAP operating loss** to reach CHF 340 million (which includes a one-off benefit of CHF 125 million from the Viatris deal), **non-GAAP operating loss** of around CHF 420 million (excluding contract revenues and the one-off benefit from the Viatris deal) – unforeseen events excluded.

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"We have already reached significant milestones in 2024. The deal with Viatris to accelerate the development of selatogrel and cenerimod brought cash and security for these assets, while retaining shareholder value. We came to an arrangement with holders of the convertible bond 2024 due for repayment in July, giving us the time we need to secure additional funding and avoid liquidity constraints. Also, the quality of our research and development engine was once again confirmed by the FDA's approval of TRYVIO (aprocitentan) and the CHMP positive opinion received for JERAYGO (the trade name of aprocitentan in Europe). This will now unlock value for Idorsia as we evaluate possible launch strategies – including potential partnership – for the first antihypertensive working on a new pathway seen in almost 40 years."

Jean-Paul continued: "Since the first launch of QUVIVIQ, more than 14 million tablets have been dispensed worldwide, with well over 150,000 patients benefiting from QUVIVIQ. We continue to believe in the huge potential offered by this product and – thanks to a long patent life – there is plenty of time for this potential to be realized. Continued innovation is essential to securing the company's future. Despite the reduction of the workforce, we continue to discover and develop new drugs with great potential in many areas of medicine. I am very confident that our vision to create an innovative, profitable, and sustainable science-based company will become a reality in the coming years."

Financial results

US GAAP results	**First Quarter**	
in CHF millions, except EPS (CHF) and number of shares (millions)	**2024**	**2023**
Net revenues	10	21
Operating expenses	20	(219)
Operating income (loss)	31	(198)
Net income (loss)	30	(212)
Basic EPS	0.17	(1.19)
Basic weighted average number of shares	179.1	178.0
Diluted EPS	0.13	(1.19)
Diluted weighted average number of shares	233.3	178.0

Net revenue of CHF 10 million in the first quarter of 2024 is the result of QUVIVIQ product sales. This compares to CHF 21 million in the first quarter of 2023, which included CHF 13.5 million sales of PIVLAZ in Japan (now assigned to Nxera Pharma as part of a transaction, more details can be found in the dedicated press release) and CHF 1 million revenue share from Johnson & Johnson related to ponesimod sales (revenue-sharing agreement now eliminated as part of the reacquisition of aprocitentan, more details can be found in the dedicated press release).

US GAAP operating expenses in the first quarter of 2024 benefitted from extraordinary income of CHF 125 million from the Viatris deal resulting in a negative expense of CHF 20 million (CHF 219 million in the first quarter of 2023), of which CHF 4 million related to cost of sales (CHF 1 million in the first quarter of 2023), CHF 33 million to R&D expenses (CHF 93 million in the first quarter of 2023), and CHF 68 million to SG&A expenses (CHF 125 million in the first quarter of 2023).

US GAAP net income in the first quarter of 2024 amounted to CHF 30 million (CHF 212 million net loss in the first quarter of 2023). The decrease of the net loss is mainly attributable to the one-off income related to the Viatris Deal but was also driven by lower operating expenses throughout all functions.

The US GAAP net income resulted in a basic net income per share of CHF 0.17 (diluted net income per share of CHF 0.13) in the first quarter of 2024, compared to a net loss per share of CHF 1.19 (basic and diluted) in the first quarter of 2023.

Non-GAAP* measures **in CHF millions, except EPS (CHF) and number of shares (millions)**	**First Quarter** **2024**	 **2023**
Net revenues	10	21
Operating expenses	(96)	(202)
Operating income (loss)	(85)	(181)
Net income (loss)	(86)	(189)
Basic EPS	(0.48)	(1.06)
Basic weighted average number of shares	179.1	178.0
Diluted EPS	(0.48)	(1.06)
Diluted weighted average number of shares	179.1	178.0

** 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 quarter of 2024 amounted to CHF 86 million: the CHF 116 million difference versus US GAAP net income was mainly due to the one-off effect of the Viatris Deal (CHF 125 million income), depreciation and amortization (CHF 4 million), and share-based compensation (CHF 4 million).

The non-GAAP net loss resulted in a net loss per share of CHF 0.48 (basic and diluted) in the first quarter of 2024, compared to a net loss per share of CHF 1.06 (basic and diluted) in the first quarter of 2023.

Viatris collaboration

In March 2024, Idorsia closed agreements with Viatris Inc. (NASDAQ: VTRS), a global healthcare company, for collaboration on the global development and commercialization of two Phase 3 assets – selatogrel and cenerimod – with Idorsia receiving an upfront payment of USD 350 million, and the right to potential development and regulatory milestone payments of up to USD 300 million, potential sales milestone payments of up to USD 2.1 billion, and potential contingent tiered royalties from mid-single- to low-double-digit percentage on annual net sales.

A joint development committee is overseeing the development of the ongoing Phase 3 programs for selatogrel and cenerimod up to regulatory approval. Idorsia will contribute up to USD 200 million in the next 3 years and transferred the dedicated personnel for both programs to Viatris.

Viatris has worldwide commercialization rights for both selatogrel and cenerimod (excluding, for cenerimod only, Japan, South Korea, and certain countries in the Asia-Pacific region). Idorsia has also granted Viatris a right of first refusal and first negotiation for certain other pipeline assets.

Convertible bonds 2024

In July 2018, the Group issued CHF 200 million of senior unsecured convertible bonds (ISIN: CH0426820350), which were due to mature on July 17, 2024. On May 6, 2024, a bondholder meeting was held, where 83.5% of the total outstanding bondholders voted in favor of amendments to the terms of the bonds. The approved bond terms include an amended conversion price of CHF 6.00, extended maturity date of January 17, 2025, and the option to call the bonds at par, in full or in part, at any time upon giving ten trading days' notice. The company has applied to the higher cantonal

composition authority and upon approval the amendments to the bond terms will become binding and effective. A consent fee of 8,000,000 Idorsia shares will be delivered through SIX SIS once the amendment of the bond terms is effective.

Financial outlook 2024

For 2024 – excluding unforeseen events – the company expects QUVIVIQ net sales of around CHF 55 million; SG&A expenses of around CHF 300 million; R&D expense of around CHF 165 million for Idorsia-led pipeline assets; non-GAAP operating expenses of up to CHF 470 million. This performance would result in a non-GAAP operating loss of around CHF 420 million (excluding contract revenues and the one-off benefit from the Viatris deal).

The company expects US GAAP operating loss for 2024 to reach CHF 340 million which includes a one-off benefit of CHF 125 million from the Viatris deal.

André C. Muller, Chief Financial Officer, commented:
"In addition to the funds already raised from our business development activities, I am confident in our ability to raise additional funding this year. We will continue to evaluate and prepare possible launch strategies – including potential partnership – for TRYVIO. The significant progress with access and availability of QUVIVIQ has started to gain traction, particularly in Europe, this will translate into higher sales in 2024. At the same time, the cost reduction initiative that took place in the latter part of 2023 is fully effective and reflected in our 2024 guidance, with significantly lower expenses. We must continue to control our costs and explore all avenues to extend our cash runway, but I see many reasons to be optimistic for the future of Idorsia."

Liquidity and indebtedness

At the end of the first quarter of 2024, Idorsia's liquidity amounted to CHF 335 million.

(in CHF millions)	March 31, 2024	Dec 31, 2023
Liquidity		
Cash and cash equivalents	335	145
Short-term deposits	-	-
Total liquidity*	**335**	**145**
Indebtedness		
Convertible loan	335	335
Convertible bond	797	796
Other financial debt	162	162
Total indebtedness	**1,293**	**1,293**

*rounding differences may occur

Commercial operations

In the first quarter of 2024, QUVIVIQ™ (daridorexant) in the US, Germany, Italy, Switzerland, Spain, UK, Canada, Austria, and France generated total product sales of CHF 10 million.

United States

Product	Mechanism of action	Indication	Commercially available since
QUVIVIQ (daridorexant) IV 25mg, 50mg tablets	Dual orexin receptor antagonist	Treatment of adult patients with insomnia, characterized by difficulties with sleep onset and/or sleep maintenance	May 2022

In the US, net sales of **QUVIVIQ® (daridorexant)** in the first quarter of 2024 reached CHF 6.5 million. This net sales number includes the QUVIVIQ copay program aimed at driving demand and product uptake, and thus does not reflect the actual number of prescriptions dispensed.

As of the end of the first quarter of 2024, more than 140,000 patients have been treated with QUVIVIQ, almost 400,000 prescriptions have been dispensed, and the product has been prescribed by more than 42,000 healthcare professionals. To begin with, the company ran a direct-to-consumer (DTC) television and digital campaign and offered a copay program. The strategy was to create a recognizable brand, enabling market access discussions. During 2023, the company made significant progress, reaching over 65% reimbursement in the commercial sector. As access increased, the commercial approach was adjusted, with the aim being to switch from a consignment model (providing substantially reduced or free prescriptions) to a payer paid model. In the first quarter of 2024, paid prescriptions accounted for 68% of the total – an increase of 36 percentage points from the same period in 2023 and of 7 percentage points from the previous quarter.

The first Medicare Part D coverage – reaching 27% of covered lives – began in January 2024, opening an entirely new channel which has the potential to substantially improve product access and paid prescriptions.

In February, there was a cyberattack on Change Healthcare (UnitedHealth Group), the largest adjudicator/processor of copay cards in the US, causing major disruption across the pharmaceutical industry, including the QUVIVIQ copay cards, with a negative impact on prescription dispensing levels. In March, the Idorsia US Market Access team put a solution in place to remedy the disruption, though the impact was still appreciable through to the end of March.

In April 2023, Idorsia filed a citizen petition (CP), urging the Drug Enforcement Administration (DEA) to deschedule the DORA class of chronic insomnia medications, based on a review of evidence from available data, including post-marketing surveillance data. Starting in 2015, the independent FDA approvals of other DORAs included a recommendation that these drug products be scheduled based on preclinical data. The CP to deschedule the DORA class outlines current scientific and medical evidence demonstrating that the DORA class has a negligible abuse profile and potential for abuse, lacks non-medical use in the community, lacks physical and psychological dependence, and therefore, should not be a scheduled class under the Controlled Substances Act.

The DEA and FDA acknowledged the CP, and the process to analyze and examine the request is moving forward. Notably, a report accompanying the FDA appropriations bill that was finalized in March 2024 informed the FDA that the process for descheduling the DORA class is a priority for Congress.

Tausif 'Tosh' Butt, President, and General Manager of Idorsia US, commented:
"I believe one of the biggest barriers to prescribing QUVIVIQ is the fact it is currently a scheduled drug. Apart from the obstacles to prescribing scheduled drugs, some payers require patients to be treated with low-cost drugs not indicated for insomnia, and others that carry black box warnings before covering QUVIVIQ. The US Congress has long supported the efforts of the FDA to address the opioid and addiction crisis, and this year it encouraged the FDA to also consider the impact of treatments for insomnia as a part of that larger public health mission. I am very hopeful for our citizen petition requesting a review of the evidence can lead to the descheduling of the DORA class of chronic insomnia medications."

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

Product	Mechanism of action	Indication	Commercially available since
TRYVIO™ (aprocitentan) 12.5mg tablets	Dual endothelin receptor antagonist	Treatment of hypertension in combination with other antihypertensive drugs, to lower blood pressure in adult patients who are not adequately controlled on other drugs	Approved Mar. 2024 Planned availability: H2 2024

On March 19, 2024, the US Food and Drug Administration (FDA) approved **TRYVIO™ (aprocitentan)** for the treatment of hypertension in combination with other antihypertensive drugs, to lower blood pressure in adult patients who are not adequately controlled on other drugs. Lowering blood pressure reduces the risk of fatal and non-fatal cardiovascular events, primarily strokes and myocardial infarctions. The recommended dosage of TRYVIO is 12.5 mg orally once daily, with or without food.

Idorsia plans to make TRYVIO available in the second half of 2024 to the millions of patients in the US whose high blood pressure is not adequately controlled by other drugs.

Further details on the approval, together with commentary from company management can be found in the dedicated press release and investor webcast available from the company corporate website.

For more information see the Full Prescribing Information including BOXED Warning (PI and Medication Guide).

Europe and Canada

Product	Mechanism of action	Indication	Commercially available
QUVIVIQ daridorexant 25mg,50mg tablets	Dual orexin receptor antagonist	Treatment of adult patients with insomnia characterised by symptoms present for at least three months and considerable impact on daytime functioning	France: Mar. 2024 Austria: Feb. 2024 UK: Oct. 2023 Spain: Sep. 2023 Switzerland: Jun. 2023 Germany: Nov. 2022 Italy: Nov. 2022
		Management of adult patients with insomnia, characterized by difficulties with sleep onset and/or sleep maintenance	Canada: Nov. 2023

QUVIVIQ (daridorexant) net sales in the first quarter of 2024 reached CHF 3.5 million in the EUCAN region.

In November 2023, treatment with daridorexant was added to the insomnia treatment guidelines for Europe. In "The European Insomnia Guideline: An update on the diagnosis and treatment of insomnia 2023", published in the *Journal of Sleep Research*, the authors note that "The introduction of DORAs has probably been the most significant recent development in the pharmacological treatment of insomnia."

In Germany, QUVIVIQ was launched in November 2022. By law, sleep medications were then subject to a 4-week prescribing limitation (Anlage III BtMG). Following a review by the Federal Joint Committee (G-BA) – the highest decision-making body of the joint self-government of physicians, dentists, hospitals, and health insurance funds in Germany – this limitation was lifted for QUVIVIQ in November 2023. This makes it the only sleep medication in Germany that can be prescribed for long-term treatment of chronic insomnia. In December 2023, the price negotiated for QUVIVIQ under the AMNOG process became effective. Following the lifting of the prescribing limitation, the company submitted a second AMNOG dossier for the long-term treatment of chronic insomnia disorder (beyond 4 weeks), reflecting the indication approved by the EMA in 2022. The progress made in Germany is reflected by the performance of QUVIVIQ on the market, with a 63% increase in demand seen in Q4 2023 (compared to Q3 2023), followed by a strong start to 2024 (February +41% compared to December 2023).

In Italy, QUVIVIQ was launched in November 2022. Currently, QUVIVIQ can only be prescribed by neurologists, psychiatrists, and specialists from sleep centers, and no sleep therapy is reimbursed. The company submitted a reimbursement dossier in June 2023 and requested the expansion of the prescriber base. The submission – detailing the efficacy and safety profile of QUVIVIQ and its estimated budget impact and cost-effectiveness in Italy – is under review, with the final outcome expected in the second half of 2024.

In Switzerland, QUVIVIQ was launched to the self-pay market in June 2023. Following the launch of QUVIVIQ, awareness has increased among all specialties, and demand has increased solidly (+32% in Q4 2023 compared to Q3 2023) ahead of reimbursement, which is expected in the summer of 2024.

In Spain, QUVIVIQ was launched to the self-pay market in September 2023. Spain represents the largest insomnia market in Europe, as was apparent in the first months of this product's availability, despite it only being launched to the self-pay market. The company is assessing the opportunity to submit a reimbursement dossier to the Spanish authorities, in order to allow equal access for all patients with chronic insomnia.

In the UK, QUVIVIQ was launched in October 2023. At the same time, technology appraisal guidance was published by the National Institute for Health and Care Excellence (NICE), allowing the transition to local access discussions and listing by healthcare boards for England, Wales, and Northern Ireland. In April 2024, the Scottish Medicines Consortium (SMC) also accepted QUVIVIQ for use within NHS Scotland. This means that the company has achieved full reimbursement throughout the UK, where QUVIVIQ is now recommended as first-line pharmaceutical treatment for patients with chronic insomnia, after, or as an alternative to, cognitive behavioral therapy for insomnia (CBT-I). The priority in the UK now, is to secure regional access.

In France, QUVIVIQ was launched in March 2024 as the first and only pharmacotherapy recommended for the treatment of chronic insomnia disorder. In January 2024, the inclusion of QUVIVIQ in both the hospital and the retail formulary list of reimbursed pharmaceutical specialties was announced in the French Official Gazette, together with the French public price. This official publication means that, with a prescription from their doctor, patients with chronic insomnia in France have access to the treatment if they meet the requirements of the EU prescribing label for QUVIVIQ. The publication follows the positive recommendation by the Transparency Committee in May 2023, recognizing QUVIVIQ as providing clinical added value.

In Canada, after being approved in April 2023, QUVIVIQ was launched in November 2023 to the private market, representing 55% of the Canadian insomnia market. The reimbursement dossier was submitted to private market payers in the third quarter of 2023, and just a few months after the submission the team had secured reimbursement for more than 60% of private market patients. The focus is now on public payers with the submission to INESSS (Institut national d'excellence en santé et en services sociaux) finalized in March 2024 and the submission to CADTH (Canada's Drug and Health Technology Agency) expected in the second quarter of 2024.

Jean-Yves Chatelan, President of Europe and Canada region, commented:
"The launch of Europe's first and only dual orexin receptor antagonist is progressing well across all markets where we have made QUVIVIQ available. Including Canada, we have expanded availability into more markets and improved the reimbursement environment beyond many expectations. With continued positive feedback from physicians and patients on the differentiated profile of QUVIVIQ, I am very optimistic that the progress we have made will now translate into many more patients benefiting from QUVIVIQ and increasing volumes advancing the region towards profitability."

For more information about QUVIVIQ in the EU, see the Summary of Product Characteristics. For more information about QUVIVIQ in Switzerland, see the Patient Information and Information for Healthcare Professionals. For more information on the marketing authorization of QUVIVIQ in Canada, see the Product Monograph.

Research & Development

Idorsia has a diversified and balanced portfolio, comprising assets developed and/or marketed by Idorsia and assets that are partner-led to maximize the value we have created. Our drug discovery engine has produced innovative drugs with the potential to transform the treatment paradigm in multiple therapeutic areas, including CNS, cardiovascular, and immunological disorders, as well as orphan diseases.

The company also has a vaccine platform for the discovery and development of glycoconjugate vaccines containing synthetic antigenic glycan molecules, with or without a carrier protein, to prevent infection.

Alberto Gimona, MD and Head of Global Clinical Development of Idorsia, commented:
"Despite a difficult period for our organization, the team has shown extraordinary commitment and made great progress with our portfolio. This is particularly evident in the successful registration of aprocitentan in the US and the positive opinion from the European Union's CHMP, with labels that reflect the value of the compound. I was also very pleased to have found a way for both selatogrel and cenerimod programs to be fully supported through the collaboration with Viatris, while maintaining our involvement in their development. I look forward to advancing the portfolio and bringing benefits to patients in many areas of medical need."

Idorsia-led portfolio

Compound Mechanism of action **Target indication**	**Status**
QUVIVIQ™ (daridorexant) Dual orexin receptor antagonist **Insomnia**	Commercially available as QUVIVIQ in the US, Germany, Italy, Switzerland, Spain, the UK, Canada, Austria, and France; approved throughout the EU
TRYVIO™ (aprocitentan) Dual endothelin receptor antagonist **Systemic hypertension in combination with other antihypertensives**	Approved as TRYVIO in the US, launch planned for H2 2024
JERAYGO™ (aprocitentan) Dual endothelin receptor antagonist **Resistant hypertension in combination with other antihypertensives**	Positive opinion from the European Committee for Medicinal Products for Human Use (CHMP) received in April 2024 – European Commission decision expected in approx. 2 months
Lucerastat Glucosylceramide synthase inhibitor **Fabry disease**	Phase 3 primary endpoint not met; open-label extension study ongoing Phase 3 focused on renal function in preparation
Daridorexant Dual orexin receptor antagonist **Pediatric insomnia**	Phase 2 in pediatric insomnia ongoing
ACT-1004-1239 ACKR3/CXCR7 antagonist **Demyelinating diseases including multiple sclerosis**	Phase 2 in preparation
Sinbaglustat GBA2/GCS inhibitor **Rare lysosomal storage disorders**	Phase 1 complete
ACT-777991 CXCR3 antagonist **Recent-onset Type 1 diabetes**	Phase 1 complete
IDOR-1117-2520 Undisclosed **Immune-mediated disorders**	Phase 1 ongoing
IDOR-1134-2831 Synthetic glycan vaccine ***Clostridium difficile* infection**	Phase 1 initiating

Daridorexant
Daridorexant is a dual orexin receptor antagonist (DORA) which blocks the binding of the wake-promoting orexin neuropeptides. Rather than inducing sleep through broad inhibition of brain activity, daridorexant only blocks the activation of orexin receptors. Daridorexant is commercially available as QUVIVIQ in the US, Germany, Italy, Switzerland, Spain, the UK, Canada, Austria, and France, and is approved throughout the EU (see "Commercial operations" above).

A post-approval study to investigate the efficacy of daridorexant in patients with insomnia and comorbid nocturia has completed recruitment and is expected to report results in mid-2024 (NCT05597020).

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 10 to <18 years with insomnia disorder (NCT05423717). The primary objective of the study is to characterize the dose-response relationship of daridorexant on objective 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 mg, 25 mg, or 50 mg daridorexant, or placebo. The study is part of a US FDA-approved Pediatric Study Plan and an EU PDCO-approved Paediatric Investigation Plan.

Aprocitentan
Aprocitentan is a once-daily, orally active, dual endothelin receptor antagonist, which inhibits the binding of ET-1 to ET_A and ET_B receptors. Aprocitentan has a low potential for drug-drug interaction and a mechanism of action suited for lowering blood pressure in adult patients whose hypertension is not adequately controlled by other drugs. On March 19, 2024, aprocitentan was approved as TRYVIO in the US, with availability planned for H2 2024. On April 25, 2024, Idorsia received a positive opinion for aprocitentan (as JERAYGO™) from the Committee for Medicinal Products for Human Use (CHMP) as a treatment of resistant hypertension. A CHMP positive opinion is one of the final steps before marketing authorization can be granted by the European Commission; a final decision is expected approximately two months after publication of the CHMP opinion.

Lucerastat
Lucerastat is an oral inhibitor of glucosylceramide synthase, offering a potential new treatment approach for all patients living with Fabry disease, irrespective of the mutation type of the GLA gene. In October 2021, the company reported that lucerastat 1000 mg b.i.d. did not meet the primary endpoint of reducing neuropathic pain during 6 months of treatment versus placebo. However, Lucerastat demonstrated a substantial reduction in levels of the Fabry disease biomarker plasma Gb3 during the treatment period, with a decrease of approximately 50% observed in plasma Gb3 in the lucerastat treatment group compared to an increase of 12% in the placebo group. Furthermore, results suggested a treatment effect on kidney function. Lucerastat was well tolerated. Analysis of the ongoing open-label extension (OLE) of the Phase 3 study corroborated the long-term effect on plasma Gb3 levels and a potential positive long-term effect on kidney function. The analysis also showed a safety and tolerability profile consistent with that observed during the 6-month randomized treatment period. The company is conducting a kidney biopsy substudy within a subset of patients currently participating in the OLE study in order to steer further development in Fabry disease.

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

Idorsia partner-led portfolio

For Idorsia, sophisticated partnerships are a way of gaining strategic access to technologies or products and fully exploiting our discovery engine and clinical pipeline. We seek suitable external project partners to maximize the value of internal innovation.

Compound Mechanism of action **Target indication**	**Partner/**status
Daridorexant Dual orexin receptor antagonist **Insomnia**	**Nxera Pharma:** license to develop and commercialize for Asia-Pacific region (excluding China) NDA submitted in Japan
Daridorexant Dual orexin receptor antagonist **Insomnia**	**Simcere:** license to develop and commercialize for Greater China region Phase 3 ongoing
Selatogrel $P2Y_{12}$ inhibitor **Acute myocardial infarction**	**Viatris:** worldwide development and commercialization rights Phase 3 "SOS-AMI" program ongoing
Cenerimod $S1P_1$ receptor modulator **Systemic lupus erythematosus**	**Viatris:** worldwide development and commercialization rights (excluding Japan, South Korea, and certain countries in the Asia-Pacific region) Phase 3 "OPUS" program ongoing
Daridorexant Dual orexin receptor antagonist **Posttraumatic stress disorder (PTSD)**	**US Department of Defense (DOD)**: Idorsia is supporting a clinical study sponsored by the US DOD to develop new therapies to treat PTSD
ACT-709478 (NBI-827104) T-type calcium channel blocker **Epileptic encephalopathy with continuous spike-and-wave during sleep (CSCW)**	**Neurocrine Biosciences:** global license to develop and commercialize Phase 2 OLE study ongoing
ACT-1002-4391 EP_2/EP_4 receptor antagonist **Immuno-oncology**	**Owkin:** global license to develop and commercialize Phase 1 in preparation

Daridorexant (Nxera Pharma)

Daridorexant is licensed to Nxera Pharma (previously known as Sosei Heptares) in the Asia-Pacific region (excluding China), and a New Drug Application (NDA) is under review with the Japanese Ministry of Health, Labor, and Welfare (MHLW).

In Japan, Idorsia has a license agreement with Mochida Pharmaceutical for the supply, co-development and co-marketing of daridorexant. All potential milestones have been assigned to Nxera.

Asia-Pacific region (excluding China): Australia, Brunei, Cambodia, Indonesia, Japan, Laos, Malaysia, Myanmar, New Zealand, Philippines, Singapore, South Korea, Thailand, Taiwan, and Vietnam.

Daridorexant (Simcere)

Daridorexant is licensed to Simcere in the Greater China region (Mainland China, Hong Kong, and Macau), and a Phase 3 study with daridorexant in Chinese patients has completed recruitment. Results are expected in June 2024 and, if the study is successful, an NDA in Mainland China is planned for the second half of 2024. An NDA is already under review with the Hong Kong Department of Health.

Selatogrel and cenerimod (Viatris)
A joint development committee from Idorsia and Viatris is overseeing the development of two ongoing Phase 3 programs up to regulatory approval.

Selatogrel is a potent, fast-acting, reversible, and highly selective $P2Y_{12}$ inhibitor being developed in a Phase 3 study (NCT04957719) for the treatment of acute myocardial infarction ("SOS-AMI") in patients with a recent history of AMI. It is intended to be self-administered subcutaneously via a drug delivery system (autoinjector).

Cenerimod is a highly selective $S1P_1$ receptor modulator, given as an oral once-daily tablet, which is being developed in a Phase 3 program known as "OPUS" (NCT05648500, NCT05672576) for the treatment of systemic lupus erythematosus (SLE).

Viatris has worldwide commercialization rights for both selatogrel and cenerimod (excluding, for cenerimod only, Japan, South Korea, and certain countries in the Asia-Pacific region).

Daridorexant (US Department of Defense)
Idorsia is supporting a clinical study sponsored by the US Department of Defense (DOD) to develop new therapies for posttraumatic stress disorder (PTSD). The Phase 2 study will evaluate the safety, tolerability, and efficacy of potential therapeutic interventions, including daridorexant, in active-duty US service members and veterans with PTSD (NCT05422612).

ACT-709478
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 being investigated in a Phase 2 open-label extension (OLE) study for the treatment of pediatric patients with epileptic encephalopathy with continuous spike-and-wave during sleep (CSCW), a rare form of pediatric epilepsy. While the blinded study did not meet the primary endpoint, ACT-709478 was generally well tolerated and Neurocrine continues to analyze the totality of data coming from the OLE study to determine the next steps.

ACT-1002-4391
Owkin has a global license to develop and commercialize ACT-1002-4391, Idorsia's novel, potent EP_2/EP_4 receptor antagonist with antitumor efficacy, to be used both as monotherapy and in combination with other oncology agents. The compound is in preparation for Phase 1 clinical pharmacology studies. Owkin will use its proprietary AI-based data-mining platform to generate clinical trial designs and to identify patients who may benefit from, and potential targets for, the compound.

Martine Clozel, MD and Chief Scientific Officer, commented:
"The way we work in research is focused on and built around innovation and our core competencies. While we had to cut back on the number of people conducting research in 2023, we have maintained this fundamental approach to our drug discovery efforts. We have taken the restructuring as an opportunity to focus on fewer key areas of research and will advance our discoveries either through our own clinical development expertise, or with the right partner, aiming to maximize the benefit for patients and Idorsia."

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

- Annual General Meeting of Shareholders on June 13, 2024
- Half-Year 2024 Financial Results reporting on July 25, 2024
- Nine-Months 2024 Financial Results reporting on October 29, 2024

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 25-year heritage of drug discovery, a broad portfolio of innovative drugs in the pipeline, an experienced team of professionals covering all disciplines from bench to bedside, and commercial operations in Europe and North America – the ideal constellation for bringing innovative medicines to patients.

Idorsia was listed on the SIX Swiss Exchange (ticker symbol: IDIA) in June 2017 and has over 750 highly qualified specialists dedicated to realizing our ambitious targets.

For further information, please contact

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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.