Media Release
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Idorsia announces capital increase to prepare for the launch of daridorexant and further fund its diversified pipeline

- **Idorsia** announces capital increase by way of an at-market rights offering with envisaged gross proceeds of approximately CHF 575 million
- The proceeds from the offering will be used to support the regulatory filing and, if approved, commercial launch of daridorexant and to fund the further development of its diversified pipeline
- Jean-Paul and Martine Clozel remain fully committed to Idorsia over the long-term and as such have committed to exercise all their subscription rights in order to maintain – at least – their current shareholding of 28.4%

Allschwil, Switzerland – October 8, 2020
Idorsia Ltd (SIX: IDIA) today announced a capital increase by way of an at-market rights offering with envisaged gross proceeds of approximately CHF 575 million. Idorsia intends to use the net proceeds from the rights offering primarily to support the regulatory filing and, if approved, commercial launch of daridorexant, its dual orexin receptor antagonist, and fund the clinical development of other late-stage compounds such as aprocitentan, clazosentan, lucerastat, cenerimod, and selatogrel as well as early-stage pipeline assets.

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"Idorsia has made a quantum leap since incorporation in 2017. With our lead compound daridorexant reporting positive results in both pivotal studies this year, we are moving into a new phase and preparing the way to bring our first product to patients. If we do our job well, daridorexant alone should be enough for us to achieve sustainable profitability within a relatively short period of time, which means our additional pipeline assets will bring significant growth opportunities. This fundraising will see us through to the launch of our first product, as well as seeing some key clinical data from late-stage assets. We are well on our way to delivering on our strategic priorities and developing Idorsia into one of Europe's leading biopharmaceutical companies, with a strong scientific core."

The strategy of Idorsia is to skilfully invest in our pipeline, following a structured approach to capital management as well as creating a sustainable revenue-generating organization in order to create value for patients, prescribers, investors, employees and our other stakeholders.

Clinical Development Pipeline
We have a diversified and mostly unencumbered portfolio of compounds in our clinical development pipeline, which we believe sets us apart from other clinical-stage biopharmaceutical companies. Our clinical development pipeline comprises a total of 12 compounds.

Compound	Mechanism of Action	Target Indication	Status
Daridorexant	Dual orexin receptor antagonist	Insomnia	Filing in preparation
Aprocitentan*	Dual endothelin receptor antagonist	Resistant hypertension management	Phase 3
Clazosentan	Endothelin receptor antagonist	Vasospasm associated with aneurysmal subarachnoid hemorrhage	Phase 3
Lucerastat	Glucosylceramide synthase inhibitor	Fabry disease	Phase 3
Selatogrel	$P2Y_{12}$ receptor antagonist	Suspected acute myocardial infarction	Phase 3 in preparation
Cenerimod	$S1P_1$ receptor modulator	Systemic lupus erythematosus	Phase 2
ACT-774312	CRTH2 receptor antagonist	Nasal polyposis	Phase 2
ACT-539313	Selective orexin 1 receptor antagonist	Psychiatric disorders	Phase 2 in preparation
Sinbaglustat	GBA2/GCS inhibitor	Rare lysosomal storage disorders	Phase 1 complete
ACT-1004-1239	—	Immunology / Cancer immunotherapy	Phase 1
ACT-1014-6470	—	Immunology	Phase 1
ACT-541478	—	CNS	Phase 1

* *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 our ACT-709478, a novel T-type calcium channel blocker, for the treatment of a rare form of pediatric epilepsy. In May 2020, Neurocrine announced plans to initiate a Phase 2 study for ACT-709478 in the second half of 2020.

The late-stage pipeline includes the following assets:

Daridorexant
Daridorexant is a dual orexin receptor antagonist developed for the treatment of insomnia. The Phase 3 registration program demonstrated statistically significant and clinically meaningful improvements in sleep and daytime functioning, that were sustained over time.

Results of the first study, investigating daridorexant doses 25 and 50 mg vs. placebo, were reported in April 2020. Compared to placebo, the study demonstrated efficacy of treatment with daridorexant on objective (as measured in a sleep lab by polysomnography) and subjective (as measured daily with a patient diary at home) sleep parameters, and on daytime functioning on 50 mg, with a trend on 25 mg, with patients reporting no morning sleepiness, no signals suggestive of rebound insomnia compared to baseline sleep parameters, and no withdrawal symptoms upon treatment discontinuation. The most frequent AEs, nasopharyngitis and headache, were balanced between arms. Somnolence was reported in 6 (1.9%) patients on placebo, 11 (3.5%) patients on daridorexant 25 mg, and 5 (1.6%) of patients on daridorexant 50 mg.

Results of the second study investigating daridorexant doses 10 and 25 mg versus placebo, were reported in July 2020. The study confirmed the findings of the first pivotal study, demonstrating

efficacy of treatment with daridorexant on objective and subjective sleep parameters. In addition, a positive trend on daytime functioning was observed on 25 mg, consistent with the results of the first study. The safety profile was consistent with the results of the first study.

The similar design of the two Phase 3 studies allowed for the two groups of 25 mg and placebo to be pooled and a pre-planned analysis to be made. The pooled analysis supports the safety and efficacy of the 25 mg dose of daridorexant.

Furthermore, an interim analysis of the ongoing 40-week extension study, once all patients had received 6 months of treatment (during the core and the extension study together), has been conducted. The study, which primarily measures the safety of long-term treatment of daridorexant and allows an exploratory analysis of the maintenance of efficacy, did not uncover new emerging safety findings, neither qualitatively nor quantitatively, while the efficacy on sleep and daytime functioning is maintained over the longer treatment duration.

In addition, a comprehensive clinical pharmacology program of 18 studies has delivered robust results to be included in the filing with health authorities.

Jean-Paul Clozel, continued:
"We believe these results make daridorexant the first non-sedative sleeping pill to demonstrate an improvement in sleep and daytime functioning as measured with a new instrument specifically developed and validated, while keeping a favorable safety profile. I'm particularly pleased that with the highest dose of daridorexant, morning sleepiness, as measured by the visual analogue scale, was comparable to placebo, something I believe is a real differentiator. We have now collected the required data and held initial discussions with the US FDA which keeps us on target for filing the New Drug Application (NDA) with the FDA around the end of 2020. This would in-turn allow for commercialization and launch – subject to approval – in the first half of 2022."

Aprocitentan

Aprocitentan is an orally active potent dual endothelin receptor antagonist, which is currently investigated in PRECISION, a Phase 3 study to demonstrate the antihypertensive effect of aprocitentan when added to standard of care for patients whose blood pressure is uncontrolled despite receiving triple antihypertensive therapy, so-called resistant hypertension. In consultation with regulatory authorities, we have designed a single study which will efficiently address both the short-term efficacy of aprocitentan and the durability of its effects in long-term treatment. The study is targeting the randomization of 600 patients in approximately 180 sites in around 20 countries and results are targeted for the second half of 2022.

Clazosentan

Clazosentan is a selective endothelin A receptor antagonist which is currently investigated in REACT, a Phase 3 study to investigate the efficacy and safety of clazosentan for the prevention of clinical deterioration due to vasospasm-related delayed cerebral ischemia in adult patients following an aneurysmal subarachnoid hemorrhage (aSAH). The Phase 3 study incorporates the learnings from the previous clazosentan program to identify patients at high risk of vasospasm and delayed cerebral ischemia, the optimal dose, the best measure to demonstrate efficacy and an optimized set of patient management guidelines to ensure patient safety. Approximately 400 patients – treated either with clipping or endovascular coiling – are being enrolled in REACT at approximately 95 sites across 15 countries. Completion of the study is targeted for the second half of 2022.

In addition, a registration program in Japan, to assess the efficacy and safety of clazosentan in reducing vasospasm and vasospasm-related morbidity and mortality events in adult patients with

aSAH, has completed recruitment in the first half of 2020. The results are on target to be announced by the end of 2020, with a rapid turnaround for filing of the dossier with the Japanese health authority.

Lucerastat

Lucerastat is an oral inhibitor of glucosylceramide synthase which offers a potential new treatment approach for patients living with Fabry disease, irrespective of mutation type. Preclinical studies have shown that lucerastat is an orally available, small molecule with rapid absorption that is widely distributed to most tissues, including the central nervous system, kidney and heart. Lucerastat is currently investigated in MODIFY, a Phase 3 study to determine the efficacy and safety of lucerastat oral monotherapy in adult patients with Fabry disease. Enrollment will be completed by the end of 2020 with 90 to 100 patients ultimately being randomized to lucerastat or placebo in a 2:1 ratio. Results of this study are targeted for the second half of 2021.

Selatogrel

Selatogrel is a potent, fast-acting, reversible, and highly-selective $P2Y_{12}$ receptor antagonist being developed for the treatment of a suspected acute myocardial infarction (AMI or heart attack) in patients with a history of AMI. It is intended to be self-administered subcutaneously via an auto-injector. This novel emergency, self-administered agent is being investigated to assess its potential to protect heart muscle at the very early phase of an AMI in the crucial time between symptom onset and first medical attention to prevent severe clinical outcomes. In consultation with health authorities, we are preparing a large (approximately 14,000 patients) Phase 3 study to evaluate the efficacy and safety of self-administered subcutaneous injection of selatogrel for the treatment of a suspected AMI in patients with a history of AMI. A special protocol agreement with the FDA has been granted and initiation of the registration study is targeted for the first half of 2021.

Cenerimod

Cenerimod is a selective sphingosine-1-phosphate 1 ($S1P_1$) receptor modulator, which potentially offers a novel approach for the treatment of systemic lupus erythematosus ("SLE"), a disease with a significant impact on patients and limited treatment options. Cenerimod is currently investigated in CARE, a multiple-dose, efficacy and safety study for the treatment of adult patients with moderately to severely active, autoantibody-positive SLE. The study is currently enrolling patients, with results targeted for the first half of 2022.

Nine-months 2020 Financial Results

Due to the timing of the fundraising the company is providing preliminary nine-month results ahead of the offering. Final financial results will be provided on October 27, 2020.

Preliminary 9 Month 2020 Figures

We expect our total liquidity to amount to CHF 816 million and our financial debt to amount to CHF 585 million, both amounts as of 30 September 2020.

Net revenue is expected to amount to CHF 66 million for the nine months ended 30 September 2020 (of which CHF 8 million is expected for the third quarter).*

US GAAP total operating expenses are expected to be in the range of CHF 350 million to CHF 360 million for the nine months ended 30 September 2020 (of which CHF 113 million to CHF 123 million are expected for the third quarter).

Non-GAAP total operating expenses are expected to be in the range of CHF 297 million to CHF 307 million for the nine months ended 30 September 2020 (of which CHF 104 million to CHF 114 million are expected for the third quarter).

André C. Muller, Chief Financial Officer, commented:

"The fundraising announced today will narrow the liquidity gap until we are able to generate our own sales revenue, at which point alternative ways to fund the company will become available to help us reach profitability. To support our commitment to transparency, we have issued these preliminary nine-month results. While we are yet to complete the forecast for full-year 2020 in the context of the nine-month results, we do believe that our US GAAP operating expenses for the full-year will now likely be around 500 million Swiss francs and non-GAAP operating expenses for the full-year will now likely be around 460 million Swiss francs – excluding unforeseen events, potential milestone payments and any payments related to the ongoing arbitration with Axovan vendors*. The lower than expected spend in 2020 is mainly the result of COVID-19 which impacted the recruitment pace in some of our late-stage pipeline studies."

** See notes to editor below for more details*

About the capital increase

The share capital of Idorsia will be increased by up to 23,800,000 new registered shares with a par value of CHF 0.05 each to be issued from Idorsia's existing authorized share capital, corresponding to up to 16.7% of Idorsia's currently issued share capital. Existing Idorsia shareholders will receive one subscription right for every Idorsia share held after close of trading on SIX Swiss Exchange on October 12, 2020 to subscribe for new shares in Idorsia to be issued in the capital increase. The new shares will be offered to the existing Idorsia shareholders at a ratio of 1 new share for every 6 subscription rights held, subject to certain restrictions based on residency and applicable securities laws. The subscription rights will neither be listed or traded and will lapse unless exercised during the rights exercise period.

The rights exercise period will start on October 13, 2020 and end on October 20, 2020, 12:00 noon CEST. The offering and listing prospectus will be published on October 8, 2020.

Shares not taken up by existing Idorsia shareholders may be offered to investors by way of public offering in Switzerland and to qualified investors by way of private placements in certain jurisdictions outside Switzerland and the United States of America (the "United States" or "US") pursuant to Regulation S of the US Securities Act of 1933, as amended (the "Securities Act") and in the United States to qualified institutional buyers as defined in Rule 144A of the Securities Act pursuant to an exemption from the registration requirements of the Securities Act.

The number of new shares for which rights have been exercised is expected to be announced on October 20, 2020, after close of trading on SIX Swiss Exchange.

The offer price for the new shares and the final number of new shares to be issued will be determined following a bookbuilding process for the shares not taken up by existing shareholders. The bookbuilding is expected to start on October 13, 2020 and is expected to end on October 21, 2020, 3:00pm CEST. The offer price and the final number of new shares to be issued is expected to be announced on or around October 21, 2020, after close of trading on SIX Swiss Exchange. It is currently expected that the listing according to the International Reporting Standard of SIX Swiss Exchange, first trading day, settlement and delivery of the new shares will be on October 23, 2020.

Idorsia's principal shareholders, Jean-Paul and Martine Clozel, remain fully committed to Idorsia over the long-term and as such have committed to exercise all their rights in order to maintain at least their

current shareholding of 28.4% in Idorsia. Jean-Paul and Martine Clozel may potentially even increase their shareholding up to 30% depending on the outcome of this offering.

Idorsia as well as Jean-Paul and Martine Clozel have agreed to a 180-day lockup period after the listing of the new shares, subject to customary exceptions.

Eligible investors can find further information here

Expected timetable of the capital increase

Date	Description
October 8, 2020	Publication of offering and listing prospectus
October 13–20, 2020 (noon CEST)	Rights exercise period
October 20, 2020 (after market close)	Announcement of rights take-up
October 13-21, 2020 (3:00pm CEST)	Bookbuilding period
October 21, 2020 (after market close)	Announcement of offer price and final number of new shares
October 23, 2020	First trading day of new shares, settlement and closing

Notes to the editor

Legal update on milestones regarding clazosentan and related ongoing arbitration
Following the demerger from Actelion and the transfer of a share purchase agreement with the former shareholders Axovan Ltd. (the "Axovan vendors"), Idorsia holds a license agreement to develop and commercialize clazosentan.

In 2018, approximately 65% of Axovan vendors (Claimants) entered an arbitration against Actelion claiming that the acquisition of Actelion by J&J and/or the demerger triggers the accelerated payment of all outstanding milestones under the license agreement. These claims are being vigorously contested by Actelion and by Idorsia, which is required pursuant to the demerger agreement to indemnify Actelion in respect of the claims.

In the first half 2020, Idorsia acquired all outstanding future milestone claims from approximately 26% of Axovan vendors at around 30% of their potential nominal value for a one-time payment of CHF 9 million. The company assessed that this transaction with non-claimants is the best estimate to assess all other vendors' claims, resulting in an accrual of CHF 24 million and a total R&D expense CHF 32 million. At this stage, it is difficult to predict the outcome of the ongoing arbitration which is substantially completed. This accrual may or may not cover the outcome of the ongoing arbitration that could result in a payment between CHF 0 and CHF 94 million.

Update on Santhera
Idorsia currently owns 1.7 million shares in Santhera. Idorsia expects to recognize in its financial statements ending 30 September 2020 contract revenue of CHF 2.4 million corresponding to the fair market value of 336,667 Santhera shares, with CHF 10 milllion of an exchangeable note not being recorded as contract revenue since its recoverability will depend on Santhera's ability to raise sufficient cash. For the nine months ended 30 September 2020, Idorsia expects to book in its financial result an unrealized loss of CHF 6.8 million corresponding to the change in the fair market value of its 1.7 million Santhera shares.

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 one of Europe's leading biopharmaceutical companies, with a strong scientific core.

Headquartered in Switzerland - a biotech-hub of Europe - Idorsia is specialized in the discovery and development of small molecules, to transform the horizon of therapeutic options. Idorsia has a broad portfolio of innovative drugs in the pipeline, an experienced team, a fully-functional research center, and a strong balance sheet – the ideal constellation to bringing R&D efforts to business success.

Idorsia was listed on the SIX Swiss Exchange (ticker symbol: IDIA) in June 2017 and has over 800 highly qualified specialists dedicated to realizing our ambitious targets.

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"manufacturer" (for the purposes of the MiFID II Product Governance Requirements) may otherwise have with respect thereto, the Shares have been subject to a product approval process by each underwriter established in the EEA, which has determined that the Shares are: (i) compatible with an end target market of retail investors and investors who meet the criteria of professional clients and eligible counterparties, each as defined in MiFID II; and (ii) eligible for distribution through all distribution channels as are permitted by MiFID II (the "Target Market Assessment"). Notwithstanding the Target Market Assessment, Distributors should note that: the price of the Shares may decline and investors could lose all or part of their investment; the Shares offer no guaranteed income and no capital protection; and an investment in the Shares is compatible only with investors who do not need a guaranteed income or capital protection, who (either alone or in conjunction with an appropriate financial or other adviser) are capable of evaluating the merits and risks of such an investment and who have sufficient resources to be able to bear any losses that may result therefrom.

The Target Market Assessment is without prejudice to the requirements of any contractual, legal or regulatory selling restrictions in relation to the offering Furthermore, it is noted that, notwithstanding the Target Market Assessment, the underwriters established in the EEA will only procure investors who meet the criteria of professional clients and eligible counterparties. For the avoidance of doubt, the Target Market Assessment does not constitute: (a) an assessment of suitability or appropriateness for the purposes of MiFID II; or (b) a recommendation to any investor or group of investors to invest in, or purchase, or take any other action whatsoever with respect to the Shares. Each distributor is responsible for undertaking its own target market assessment in respect of the Shares and determining appropriate distribution channels.

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