



Neurocrine Biosciences Exercises Option to License Idorsia's Novel Treatment for Rare Pediatric Epilepsy

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Neurocrine Biosciences to Develop and Commercialize ACT-709478, a Clinical Stage Selective T-type Calcium Channel Blocker for the Treatment of a Rare Pediatric Epilepsy - Phase II Study of ACT-709478 Planned for the Second Half of 2020

Idorsia Receives a \$45 Million Upfront Payment in Cash

Idorsia Will Also Be Entitled to Potential Development and Regulatory Milestone Payments up to \$365 Million and Tiered Royalties on Net Sales

SAN DIEGO and ALLSCHWIL, Switzerland, May 11, 2020 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) and Idorsia Ltd (SIX: IDIA) today announced, following Investigational New Drug (IND) application acceptance by the U.S. Food and Drug Administration (FDA), Neurocrine Biosciences has exercised its option to license the global rights of Idorsia's ACT-709478. Neurocrine Biosciences plans to initiate a Phase II study with this potent, selective, orally-active and brain penetrating T-type calcium channel blocker for the treatment of a rare pediatric epilepsy in the second half of 2020.



Kevin Gorman, Ph.D., Chief Executive Officer of Neurocrine Biosciences, commented:

"We are pleased with the FDA's acceptance of ACT-709478, a selective T-type calcium channel inhibitor, and we look forward to advancing this program into Phase II to potentially help children with a rare pediatric epilepsy. This collaboration demonstrates Neurocrine Biosciences' growing commitment in epilepsy and enhances our capabilities in precision medicine by targeting the underlying mechanism of disorders."

Martine Clozel, MD and Chief Scientific Officer of Idorsia, commented:

"Our drug discovery efforts have produced a diverse pipeline of innovative compounds, addressing different diseases where treatment options are either non-existent or unsatisfactory. If the efficacy of our selective T-type calcium channel blocker seen in preclinical models is confirmed in children with rare pediatric epilepsy, it could transform the life of children with this disease. When we discover a new drug that shows promise in an indication where we have the necessary expertise, we will vigorously pursue the development ourselves. Sometimes we believe more value can be created by working with a partner. This is one such example where Neurocrine Biosciences can bring their exceptional expertise in the development of innovative drugs for neurological disorders."

About the license and collaboration agreement

In 2019, Neurocrine Biosciences paid a \$5 million upfront fee to Idorsia for the option rights to ACT-709478 and a preclinical research collaboration. In May 2020, upon Investigational New Drug (IND) application acceptance by the U.S. Food and Drug Administration (FDA), Neurocrine Biosciences exercised the option to license

ACT-709478. The exercise of the option triggered an upfront payment of \$45 million in cash from Neurocrine Biosciences to Idorsia. In addition, Neurocrine Biosciences will provide an incremental \$7 million in funding to Idorsia as part of the research collaboration to discover, identify and develop additional novel T-type calcium channel blockers.

The agreement is subject to the following terms:

- **ACT-709478 milestones:** In addition to the up-front payment, Idorsia may also receive up to \$365 million in additional development and regulatory milestone payments. Furthermore, Idorsia may also be entitled to one-time commercial payments based on sales thresholds.
- **ACT-709478 royalties:** Idorsia will have the right to receive a tiered royalty ranging from the low double-digits to upper teen percentage in the US and a tiered royalty at slightly lower rates outside the US based upon aggregate global net sales.
- **Preclinical research collaboration:** The parties will work together to identify novel T-type channel blockers and explore their use in potential new disease states. Idorsia may be entitled to additional development, regulatory and commercial milestones as well as tiered royalties on annual sales for each product included in the research collaboration.

About ACT-709478

ACT-709478 is a potent, selective, orally-active and brain penetrating T-type calcium channel blocker in development for epilepsy. A Phase I clinical trial was completed in healthy adult subjects in 2019. The IND application was accepted by the FDA on April 30, 2020. A Phase 2 study in a rare pediatric epilepsy is planned for the second half of 2020.

Idorsia has received Rare Pediatric Disease designation from the U.S. FDA for ACT-709478 for the treatment of a rare pediatric epilepsy. The FDA grants Rare Pediatric Disease designation for diseases that primarily affect children ages 18 years or younger and fewer than 200,000 persons in the U.S.

About Neurocrine Biosciences

Neurocrine Biosciences is a neuroscience-focused, biopharmaceutical company with 28 years of experience discovering and developing life-changing treatments for people with serious, challenging and under-addressed neurological, endocrine and psychiatric disorders. The company's diverse portfolio includes FDA-approved treatments for tardive dyskinesia, Parkinson's disease and endometriosis* and clinical development programs in multiple therapeutic areas including a gene therapy for Parkinson's disease, chorea in Huntington disease, congenital adrenal hyperplasia, epilepsy, uterine fibroids* and polycystic ovary syndrome*. Headquartered in San Diego, Neurocrine Biosciences specializes in targeting and interrupting disease-causing mechanisms involving the interconnected pathways of the nervous and endocrine systems. For more information, visit neurocrine.com, and follow the company on [LinkedIn](https://www.linkedin.com/company/neurocrine). (*in collaboration with AbbVie)

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.

Neurocrine Biosciences Forward-Looking Statements

In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements related to the benefits to be derived from the agreement with Idorsia; our potential milestone and royalty payments to Idorsia; the ability to discover, identify and develop additional novel T-type calcium channel blockers and their use in potential new disease states; and the timing of completion of our clinical, regulatory, and other development activities. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are: risks and uncertainties associated with the scale and duration of the COVID-19 pandemic and resulting global, national, and local economic and financial disruptions; risk and uncertainties related to any COVID-19 quarantines, shelter-in-place and similar government orders that are currently in place or that may be put in place in the future, including the impact of such orders on our business operations and the business operations of the third parties on which we rely; risks that the FDA or other regulatory authorities may make adverse decisions regarding ACT-709478; risks that clinical development activities may not be completed on time or at all or may be delayed for regulatory, manufacturing, or other reasons, may not be successful or replicate previous clinical trial results, may fail to demonstrate that our product candidates are safe and effective, or may not be predictive of real-world results or of results in subsequent clinical trials; risks and uncertainties relating to competitive products and technological changes that may limit demand for a product candidate; risks that the benefits of the agreement with Idorsia may never be realized; risks that our product candidates may be precluded from commercialization by the proprietary or regulatory rights of third parties, or have unintended side effects, adverse reactions or incidents of misuse; and other risks described in the Company's periodic reports filed with the Securities and Exchange Commission, including without limitation the Company's quarterly report on Form 10-Q for the quarter ended March 31, 2020. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.

Idorsia Forward-Looking Statements

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.

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