Neurocrine Biosciences and Idorsia Amend Option Agreement to License Novel Treatment for Rare Pediatric Epilepsy

January 10, 2020

Neurocrine Biosciences Owns Option to Exclusively License ACT-709478, a Clinical Stage Selective T-type Calcium Channel Blocker for the Treatment of Epilepsy

Idorsia to Receive $45 Million Upfront Upon Exercise of the Option and up to $365 Million in Potential Development and Regulatory Milestone Payments

SAN DIEGO and ALLSCHWIL, Switzerland, Jan. 10, 2020 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) and Idorsia Ltd. (SIX: IDIA) announced an amendment to the agreement that was originally signed in 2019 granting Neurocrine Biosciences an option to license ACT-709478, a potent, selective, orally-active, and brain penetrating T-type calcium channel blocker, in clinical development for the treatment of a rare pediatric epilepsy. The option also includes a research collaboration to discover, identify and develop additional novel T-type calcium channel blockers.

"We are excited to leverage the scientific expertise of Idorsia in T-type calcium channel inhibition to potentially advance a Phase II ready compound to help people suffering from epilepsy. In addition to the treatment of epilepsy, the modulation of the calcium channel may be useful for the treatment of other disorders such as essential tremor and pain," said Kevin Gorman, Ph.D., Chief Executive Officer of Neurocrine Biosciences. "This option agreement enhances our commitment to utilizing precision medicine and differentiated mechanisms of action to develop a product portfolio capable of treating patients impacted by different forms of epilepsy."

Option and License Agreement Financial Terms

In 2019, Neurocrine Biosciences paid a non-refundable $5 million upfront fee to Idorsia for the option rights to ACT-709478 and a preclinical research collaboration. Furthermore, Neurocrine has agreed to cover additional costs as part of the IND application. The agreement is subject to the following terms upon Neurocrine Biosciences exercise of the option:

- **Option Exercise:** Upon Investigational New Drug (IND) application acceptance by the U.S. Food and Drug Administration (FDA), expected in mid-2020, Neurocrine Biosciences will have 30 days to exercise the option to license ACT-709478. If the option is exercised by Neurocrine Biosciences, Idorsia will receive an upfront payment of $45 million in cash. In addition, Neurocrine Biosciences will provide an incremental $7 million in funding to Idorsia as part of the research collaboration.
- **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 U.S. and a tiered royalty at slightly lower rates outside the U.S. 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 and T-type Calcium Channel Blockers

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. A Phase II study in a rare pediatric epilepsy is planned in 2H 2020.

About Neurocrine Biosciences

Neurocrine Biosciences (Nasdaq: NBIX) is a neuroscience-focused, biopharmaceutical company with more than 25 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 and endometriosis* and clinical development programs in multiple therapeutic areas including 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. *(*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 750 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 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 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 that the benefits of the agreement with Idorsia may never be realized; 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 September 30, 2019. 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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SOURCE Neurocrine Biosciences, Inc.

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