Neurocrine Biosciences and Xenon Pharmaceuticals Provide Regulatory Update on Ongoing Collaboration to Develop First-In-Class Treatment for Epilepsy

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U.S. Food and Drug Administration (FDA) Requests Additional Non-Clinical Data to Support Dose Justification Before Initiation of a Phase II Clinical Trial with NBI-921352 in Pediatric SCN8A-DEE Patients

FDA Grants Rare Pediatric Disease Designation for NBI-921352 for the Treatment of SCN8A-DEE

SAN DIEGO and BURNABY, British Columbia, Oct. 08, 2020 (GLOBE NEWSWIRE) -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) and Xenon Pharmaceuticals Inc. (Nasdaq: XENE) today provided an update on the ongoing collaboration for the clinical development of NBI-921352, previously known as XEN901. Neurocrine Biosciences has an exclusive license to NBI-921352, a clinical stage selective Nav1.6 sodium channel inhibitor with potential in SCN8A developmental and epileptic encephalopathy syndrome (SCN8A-DEE), a rare pediatric epilepsy, and other forms of epilepsy, including focal epilepsy. The U.S. Food and Drug Administration (FDA) provided feedback on an Investigational New Drug (IND) application submitted by Neurocrine Biosciences in support of a Phase II clinical trial in pediatric SCN8A-DEE patients. As part of its review of the IND, the FDA is requesting additional non-clinical data to support dose justification in this pediatric study. Neurocrine Biosciences and Xenon will engage with the FDA to address the feedback received with the goal of initiating a Phase II clinical trial in 2021. In parallel with this interaction, Neurocrine Biosciences is advancing clinical plans to develop NBI-921352 for the treatment of adult focal epilepsy. In addition, the FDA recently granted Rare Pediatric Disease Designation for NBI-921352 for the treatment of SCN8A-DEE.

“We expect to engage with the FDA in the near term to discuss their request for additional non-clinical data to enable a pediatric trial in SCN8A-DEE patients. In parallel, we are continuing to develop plans to study NBI-921352 in patients with adult focal epilepsy. We are committed to working with the FDA to address their feedback in a timely manner, with the goal of initiating a Phase II pediatric clinical trial in 2021,” said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences.

“The recent Rare Pediatric Disease Designation from the FDA underscores that SCN8A-DEE is a devastating pediatric epilepsy, with a lack of approved treatments, that results in serious, life-threatening seizures and neurodevelopmental impairment, further validating our ‘precision medicine’ approach to develop treatments for pediatric epilepsies. We are now working with the team at Neurocrine Biosciences to respond to the FDA’s request for information and also to support the clinical development plans for NBI-921352 in adult focal epilepsy,” said Dr. Simon Pimstone, Xenon’s Chief Executive Officer.

Pursuant to the Collaboration Agreement, upon FDA acceptance of an IND for NBI-921352 in either SCN8A-DEE or another major indication, Xenon is eligible to receive a milestone payment of either $25 million or $10 million, respectively, with 55% of the amount in the form of an equity investment in Xenon at a 15% premium to Xenon’s 30-day trailing volume weighted average price at that time.

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, endometriosis* and uterine fibroids*, with three pivotal and five mid-stage clinical programs in multiple therapeutic areas. 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 Xenon Pharmaceuticals Inc.
Xenon Pharmaceuticals is a clinical stage biopharmaceutical company committed to developing innovative therapeutics to improve the lives of patients with neurological disorders. Xenon is advancing a novel product pipeline of neurology therapies to address areas of high unmet medical need, with a focus on epilepsy. For more information, please visit www.xenon-pharma.com.

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 our collaboration with Xenon Pharmaceuticals Inc., Neurocrine Biosciences’ expectations with regard to its interactions and communications with the FDA, the timing of completion of our clinical, regulatory, and other development activities, and the potential to receive a priority review voucher. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are: our future financial and operating performance; the impact of the COVID-19 pandemic and efforts to mitigate its spread on our business; 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 related to the development of our product candidates; risks associated with our dependence on third parties for development and manufacturing activities related to our product candidates, and our ability to manage these third parties; risks that the FDA or other regulatory authorities may make adverse decisions regarding our product candidates; risks that clinical development activities may not be completed on time or at all, or may be delayed for regulatory, manufacturing, COVID-19 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 potential benefits of the agreements with our collaboration partners 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 our periodic reports filed with the SEC, including without limitation our quarterly report on Form 10-Q for the quarter ended June 30, 2020. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.

Xenon Pharmaceuticals' Forward-Looking Statements

This press release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and the Private Securities Litigation Reform Act of 1995 and Canadian securities laws. These forward-looking statements are not based on historical fact, and include statements regarding the timing of and results from clinical trials and other development activities, including those related to NBI-921352 and the other pre-clinical compounds covered by our collaboration with Neurocrine Biosciences; the potential efficacy, safety profile, future development plans, addressable market, regulatory success and commercial potential of NBI-921352 and the other pre-clinical compounds covered by our collaboration with Neurocrine Biosciences; the anticipated initiation of future clinical trials for NBI-921352 and the other pre-clinical compounds covered by our collaboration with Neurocrine Biosciences; our ability to achieve milestones in our collaboration with Neurocrine Biosciences and our other development programs; and the timing and results of our and our collaborators' interactions with regulators. These forward-looking statements are based on current assumptions that involve risks, uncertainties and other factors that may cause the actual results, events or developments to be materially different from those expressed or implied by such forward-looking statements. These risks and uncertainties, many of which are beyond our control, include, but are not limited to: the impact of the COVID-19 pandemic on our and our collaborators' business, research and clinical development plans and timelines and results of operations, including impact on our clinical trial sites, collaborators, and contractors who act for or on our behalf, may be more severe and more prolonged than currently anticipated; clinical trials may not demonstrate safety and efficacy of any of our or our collaborators' product candidates; any of our or our collaborators' product candidates may fail in development, may not receive required regulatory approvals, or may be delayed to a point where they are not commercially viable; we may not achieve additional milestones in our proprietary or partnered programs; regulatory agencies may not permit certain of our product candidates to advance directly into a Phase 2 or later clinical trials, may impose additional requirements or delay the initiation of clinical trials; regulatory agencies may be delayed in reviewing, commenting on or approving any of our or our collaborators' clinical development plans as a result of the COVID-19 pandemic, which could further delay development timelines; the impact of competition; the impact of new or changing laws and regulations; adverse conditions in the general domestic and global economic markets; as well as the other risks identified in our filings with the Securities and Exchange Commission and the securities commissions in British Columbia, Alberta and Ontario. These forward-looking statements speak only as of the date hereof and we assume no obligation to update these forward-looking statements, and readers are cautioned not to place undue reliance on such forward-looking statements.

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