Neurocrine Biosciences Presents Demographic and Clinical Characteristics Data of Pediatric Patients with SCN8A-Related Epilepsies at AES 2022

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SAN DIEGO, Dec. 2, 2022 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX), a leading neuroscience-focused biopharmaceutical company, today reported demographic and clinical characteristics data from genetic screening-based studies of children with *SCN8A*-related epilepsies. Data was collected from more than 17,000 patients through Invitae Corporation's Behind the Seizure® Program, of which Neurocrine Biosciences is a sponsor. The clinical characteristics of patients with pathogenic or likely pathogenic *SCN8A* variants were evaluated to assess the heterogeneity in the seizures and development of children with *SCN8A*-related epilepsies. These data (Poster #2.099: Demographic and Clinical Characteristics of Pediatric Patients with *SCN8A*-related Epilepsies: Results from a No-Charge Epilepsy Gene Panel) will be shared at the AES 2022 Annual Meeting in Nashville, Tennessee, December 2–6, 2022. (link)

"These analyses uncover the phenotypic spectrum of *SCN8A* variants and high seizure burden experienced by children with these previously undiagnosed, rare epilepsies," said Eiry W. Roberts, M.D., Chief Medical Officer. "We're proud to be a sponsor of Invitae's Behind the Seizure® program, which provides no-charge genetic testing for children under eight years old who have had one or more unprovoked seizures, including those with *SCN8A*-related epilepsies. We are conducting a Phase 2 clinical study of NBI-921352, an investigational, selective sodium channel inhibitor as a potential adjunctive therapy in children and young adults with SCN8A developmental and epileptic encephalopathy (SCN8A-DEE)."

The poster presents analyses of genetic testing data from 17,843 children under 8 years old and with ≥ 1 unprovoked seizure who participated in the Behind the Seizure® program. Data analysis demonstrated that 36 (0.2%) of the patients had an *SCN8A* variant that was classified as pathogenic (P) or likely pathogenic (LP). Of 24 unique P/LP *SCN8A* variants identified, 14 were classified as pathogenic and 10 were likely pathogenic. In patients with a P/LP *SCN8A* variant, mean (± SD) age at seizure onset was 1.4 (± 1.4) years and mean age at testing was 2.1 (± 2.4) years. The most common seizure type reported was generalized onset motor (50.0% of patients), followed by focal onset (30.6%) and generalized onset nonmotor (absence) seizures (13.9%). The most common developmental delays reported were language delays (30.6%), limited or absent speech (22.2%), and intellectual disability/motor development delays (16.7%). Over half (58.3%) of patients were reported to have experienced ≥ 1 convulsive seizure in the last month and 44.5% of patients had ≥ 1 prolonged (> 5 min) seizure over the past six months. At screening, 77.8% of patients were reported to be taking 1 to 3 antiseizure medications (ASMs) and almost one-fifth (19.4%) had previously discontinued 1 to 3 ASMs.

Neurocrine Biosciences is a sponsor of, and a participant in, the Behind the Seizures® Program Scientific Exhibit at AES 2022, where 7 companies will be presenting 30 scientific posters that highlight how the Behind the Seizures® Program is transforming genetic epilepsy diagnoses and enhancing care pathways using state-of-the-art research. The Behind the Seizures® Program Scientific Exhibit will be held on Sunday, December 4 in the Music City Center, (Room 207 C/D, 2nd floor) from 8:00am–11:00am CT.

About NBI-921352 and the Phase 2 KAYAK™ Study
NBI-921352 is an investigational, selective sodium channel 1.6 inhibitor ($Na_v1.6$) currently under development for the potential treatment of SCN8A developmental and epileptic encephalopathy (SCN8A-DEE) in children and adults. Neurocrine Biosciences is conducting the KAYAK™ Phase 2 Study to assess the efficacy, safety, tolerability, and pharmacokinetics of NBI-921352 as an adjunctive therapy in children and young adults with SCN8A-DEE. For more information about this study, please visit KayakStudy.com or ClinicalTrials.gov.

Following completion of the KAYAK™ Study, eligible participants may have the option to enroll in an open-label extension study to evaluate the long-term safety and tolerability of NBI-921352 in patients with SCN8A-DEE. For more information about this open-label study, please visit ClinicalTrials.gov.

Neurocrine Biosciences has received Orphan Drug Designation and Rare Pediatric Disease Designation from the U.S. Food and Drug Administration (FDA) for NBI-921352 in SCN8A-DEE.

About SCN8A-DEE
SCN8A developmental and epileptic encephalopathy (SCN8A-DEE) is a rare pediatric epilepsy syndrome associated with genetic mutations of the *SCN8A* gene. It is characterized by early onset seizures, developmental delay, cognitive impairment, and other medical challenges. Seizures begin at a median age of four months and are highly refractory to currently available antiseizure medication. Over 90% of children with SCN8A-DEE are non-verbal, and half are non-ambulatory. Children living with rare pediatric epilepsies, such as SCN8A-DEE, are at higher risk of sudden unexpected death in epilepsy (SUDEP). There are currently no approved therapies for this form of pediatric epilepsy.

Behind the Seizure® Program
More than 50% of epilepsies have some genetic basis. Developmental and epileptic encephalopathies (DEEs) can be primarily attributed to genetic

causes, including pathogenic mutations in the *SCN8A* gene. Identifying the potential genetic underpinnings of different forms of epilepsy could support the development of more precision treatments.

To support research into the genetics of epilepsy, Neurocrine Biosciences is a sponsor of Invitae's Behind the Seizure® program in the U.S. and Canada. This program provides free access to comprehensive testing for epilepsy-related genetic variations to any child under eight years old who has had an unprovoked seizure.

About Neurocrine Biosciences
Neurocrine Biosciences is a leading neuroscience-focused, biopharmaceutical company with a simple purpose: to relieve suffering for people with great needs, but few options. We are dedicated to discovering and developing life-changing treatments for patients with under-addressed neurological, neuroendocrine, and neuropsychiatric disorders. The company's diverse portfolio includes FDA-approved treatments for tardive dyskinesia, Parkinson's disease, endometriosis* and uterine fibroids*, as well as over a dozen mid- to late-stage clinical programs in multiple therapeutic areas. For three decades, we have applied our unique insight into neuroscience and the interconnections between brain and body systems to treat complex conditions. We relentlessly pursue medicines to ease the burden of debilitating diseases and disorders, because you deserve brave science. For more information, visit neurocrine.com, and follow the company on LinkedIn, Twitter, and Facebook. (**in collaboration with AbbVie*).

Forward-Looking Statement
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 products and product candidates, as well as from our collaboration with Invitae's Behind the Seizure® program. 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; risks related to the development of our product candidates; risks that the FDA or other regulatory authorities may make adverse decisions regarding our products or product candidates; risks that our products, and/or 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; risks associated with potential generic entrants for our products; 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 and uncertainties associated with the scale and duration of the COVID-19 pandemic and resulting global, national, and local economic and financial disruptions; 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 September 30, 2022. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof.

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