Invitae's Real-World Ciitizen Data Utilized in Praxis Precision Medicines' PRAX-222 IND Filing

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First use of Ciitizen platform as source of real-world data in regulatory filing

SAN FRANCISCO and BOSTON, Sept. 20, 2022 (GLOBE NEWSWIRE) -- Invitae (NYSE: NVTA) and Praxis Precision Medicines, Inc. (NASDAQ: PRAX), today announced that real-world clinical insights from Invitae's Ciitizen platform were utilized as natural history data to support the submission of Praxis' Investigational New Drug (IND) application for PRAX-222 for the treatment of pediatric patients with early-onset SCN2A developmental and epileptic encephalopathy (DEE). Praxis announced earlier this month that the U.S. Food and Drug Administration (FDA) cleared the IND application for the initial dose cohort for the PRAX-222 EMBRAVE clinical study.

For many well-established diseases, natural history studies and other rich data sets are available to support regulatory interactions and IND applications. For many rare diseases, including certain severe genetic pediatric epilepsies such as SCN2A-DEE, natural history studies are not yet available to document the high disease burden and significant unmet medical need. In addition, the usual method of collecting these data by having patients seen at many geographically dispersed sites is cost intensive, time consuming and not well suited to rare diseases. Invitae's Ciitizen platform enables the rapid and comprehensive collection and analysis of medical history data, which supports understanding of the patient population and disease severity, may be used as natural history data for regulatory submissions, and can inform protocol design and inclusion and exclusion criteria for clinical studies.

"The comprehensive real-world clinical evidence generated through Invitae's Ciitizen platform was a critical component to the PRAX-222 IND application, integrating a significant amount of natural history data in a highly efficient manner to help bring PRAX-222 one step closer to SCN2A-DEE patients," said Steven Petrou, Ph.D., co-founder and chief scientific officer of Praxis. "In order to make real progress and offer hope to patients living with SCN2A-DEE and their caregivers, it requires a committed ecosystem and a community willing to consider innovative approaches to drug development. We look forward to our continued partnership with Invitae for PRAX-222 and for other precision medicines targeting rare genetic epilepsies with high unmet need."

The data, collected on behalf of SCN2A-DEE patients or their parents/guardians, is de-identified and shared with their consent, and represent the richest aggregation of real-world clinical evidence for SCN2A-DEE patients. The data generated by Invitae's Ciitizen platform is comprehensive, leveraging the HIPAA right of access to gather full medical records, longitudinally, from all of the patients' sites of care. This approach addresses many of the limitations of other data sources, such as de-identified provider electronic medical record and claims data, that were raised by the FDA in recent draft guidance documents on the use of real-world data in regulatory submissions. This novel data collection and extraction model also addresses many of the logistical, financial, and methodological limitations of the site-based natural history studies, by rapidly enrolling a diverse and representative sample of patients directly without the need to engage with sites for recruitment and data collection. Additionally, the real-world data set underlying this natural history study provides a unique data collection and sharing model wherein the patients, patient advocacy groups and other researchers all have access to the data collected. Patients have complete access to the records for their own use and are also able to remain involved and informed about the research throughout the study, highlighting the benefits of this unique patient-centered research model.

"This is, to my knowledge, the first instance of a patient-mediated medical records platform being utilized as the primary natural history data source for a successful IND filing with the FDA," stated Alexandra Berk, medical affairs director at Invitae. "It's an important development in terms of the FDA's openness to include novel data sources as part of the regulatory process."

"We are thrilled to partner with Praxis and see how our Ciitizen platform's high quality data can move the needle forward for patients living with this rare, genetic epilepsy," said Robert Nussbaum, M.D., chief medical officer of Invitae. "Our goal is to advance the understanding of SCN2A-DEE and other diseases and utilize these data to bring new therapies to market faster. Ciitizen is a compelling platform for rapidly gathering both genotypic and phenotypic data with patient consent and engagement for natural history studies for rare diseases, data that are essential to regulatory processes and for developing new therapies."

Watch a recorded webinar with speakers from Invitae and Praxis describing this natural history data in more detail here. To learn more about Invitae's Ciitizen platform or to request access to the consented real-world data for research, email partners@invitae.com.

About PRAX-222
PRAX-222 is an ASO designed to selectively decrease SCN2A gene expression, directly targeting the underlying cause of early-seizure-onset SCN2A-DEE to treat seizures and other symptoms in patients with gain-of-function SCN2A mutations. In-vitro studies of PRAX-222 have demonstrated reduction in both SCN2A gene expression and protein levels. In-vivo, PRAX-222 has

demonstrated significant, dose-dependent reduction in seizures, improvement in behavioral and locomotor activity and increased survival in SCN2A mouse models, with potential to be the first disease-modifying treatment. PRAX-222 has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPD) from the FDA, and ODD from the European Medicines Agency (EMA) for the treatment of SCN2A-DEE. The PRAX-222 program is ongoing under a collaboration with Ionis Pharmaceutics, Inc. (NASDAQ: IONS) and RogCon, Inc. To learn more about SCN2A-DEE and the EMBRAVE study, please visit https://scn2a.com/.

About Invitae
Invitae Corporation (NYSE: NVTA) is a leading medical genetics company, whose mission is to bring comprehensive genetic information into mainstream medicine to improve healthcare for billions of people. Invitae's goal is to aggregate the world's genetic tests into a single service with higher quality, faster turnaround time and lower prices. For more information, visit the company's website at invitae.com.

About Praxis
Praxis Precision Medicines is a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for CNS disorders characterized by neuronal excitation-inhibition imbalance. Praxis is applying insights from genetic epilepsies to both rare and more prevalent neurological disorders, using our understanding of shared biological targets and circuits in the brain. Praxis has established a broad portfolio with multiple programs, including product candidates across movement disorders, epilepsy and psychiatric disorders, with four clinical-stage product candidates. For more information, please visit www.praxismedicines.com and follow us on LinkedIn and Twitter.

Invitae Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to the benefits of the data generated through the company’s Ciitizen platform. Forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially, and reported results should not be considered as an indication of future performance. These risks and uncertainties include, but are not limited to: the company's history of losses; the company's ability to compete; the company's failure to manage growth effectively; the company's need to scale its infrastructure in advance of demand for its tests and to increase demand for its tests; the company's ability to use rapidly changing genetic data to interpret test results accurately and consistently; security breaches, loss of data and other disruptions; laws and regulations applicable to the company's business; and the other risks set forth in the company's filings with the Securities and Exchange Commission, including the risks set forth in the company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2022. These forward-looking statements speak only as of the date hereof, and Invitae Corporation disclaims any obligation to update these forward-looking statements.

Praxis Precision Medicines Forward-Looking Statements
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995 and other federal securities laws, including express or implied statements regarding Praxis’ future expectations, plans and prospects, including, without limitation, statements regarding the development of our product candidates, including the design of our clinical trials and the treatment potential of our product candidates. The express or implied forward-looking statements included in this press release are only predictions and are subject to a number of risks, uncertainties and assumptions, including, without limitation: uncertainties inherent in clinical trials; the expected timing of submissions for regulatory approval or review by governmental authorities; regulatory approvals to conduct trials;; and other risks concerning Praxis’ programs and operations as described in its Quarterly Report on Form 10-Q for the quarter ended June 30, 2022 and other filings made with the Securities and Exchange Commission. Although Praxis’ forward-looking statements reflect the good faith judgment of its management, these statements are based only on information and factors currently known by Praxis. As a result, you are cautioned not to rely on these forward-looking statements. Any forward-looking statement made in this press release speaks only as of the date on which it is made. Praxis undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future developments or otherwise.

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