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Target ALS Launches Biomarker Study with Academic, Industry and Philanthropic Partners

Unprecedented Effort Aims to Accelerate Testing of New ALS Therapies

NEW YORK CITY, N.Y. (April 19, 2018) —Target ALS today announced the launch of a new study that connects the CReATe Consortium (Clinical Research in ALS and Related Disorders for Therapeutic Development), the Muscular Dystrophy Association and 10 pharmaceutical companies to validate the most promising ALS biomarker candidates, and immediately make all results broadly available to the worldwide ALS research community.

"By bringing academic and industry partners together, and sharing all study results freely and immediately, Target ALS is continuing to provide the tools necessary to conduct more efficient and accurate ALS clinical trials with the ultimate goal of fast-tracking effective treatments," said Dr. Manish Raisinghani, Target ALS Chief Executive Officer.

Amyotrophic lateral sclerosis (ALS) is a devastating disease for patients and their families. While scientists have made great strides in understanding the basic biology of ALS, effective treatments for the disease remain elusive. Potential therapies that show promise in the lab continue to fail at shockingly high rates in clinical trials. The development of well-characterized biomarkers will improve the performance of clinical trials and help accelerate drug development across the field.

"This partnership with Target ALS and other funding agencies, along with the engagement of industry partners, is critical to advancing the identification and validation of promising biomarkers," said Dr. Michael Benatar, Director of the CReATe Consortium, and Executive Director of the ALS Center at the University of Miami.

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The study will take a series of already promising biofluid biomarker candidates and provide the rigorous validation necessary to implement them in upcoming clinical trials. The candidates so far include urinary p75 neurotrophin receptor extracellular domain (p75ECD); blood and cerebrospinal fluid (CSF) phosphorylated neurofilament heavy (pNfH); and blood and CSF neurofilament light (NfL).

The patient samples and clinical information are collected by CReATe Consortium investigators at 15 academic institutions. CReATe is funded primarily by NCATS and NINDS as part of NIH's (National Institutes of Health) Rare Diseases Clinical Diseases Research Network, and with additional support from the ALS Association and Target ALS to enhance the CReATe Biorepository. The biomarker data from these validation experiments will be made available to ALS researchers worldwide without embargo or reach-through on intellectual property.

The study is led by Dr. Benatar and Dr. Lyle Ostrow from Johns Hopkins University. Dr. Ostrow also directs the Target ALS Multicenter Postmortem Tissue Core. "The formation of this cross-sector, multi-institutional coalition is a first step toward even greater collaboration, cooperation and coordination among industry, academic and philanthropic partners in the ALS clinical research space," Dr. Ostrow added.

In addition to Target ALS, other funders include the NIH (through its support for the CReATe Consortium) and the Muscular Dystrophy Association. Industry partners include: Amgen, Biogen, Denali Therapeutics, Genentech, Merck & Co., Sanofi Genzyme, Takeda Pharmaceutical Company Ltd and Teva Pharmaceutical Industries.

About amyotrophic lateral sclerosis

ALS is a disease of the parts of the central nervous system that control voluntary muscle movement. In ALS patients, motor neurons (nerve cells that control muscle cells) gradually lose function; the result is that the muscles they control become weak and eventually nonfunctional. ALS usually strikes in late middle age or later, although it also occurs in children, young adults and older individuals. The majority of cases of ALS are sporadic, meaning there is no known family history of the disease and the cause is unknown. However, about 10 percent of cases are familial, and result from mutation of specific disease genes. Life expectancy typically is three to five years after diagnosis, and there is no cure.

About Target ALS

Founded by Daniel L. Doctoroff, Target ALS is a pioneering medical research foundation dedicated to accelerating the discovery and development of effective treatments for patients with ALS. The Foundation offers all of the critical resources to researchers that are essential for accelerated drug discovery, including robust financial support and access to cutting-edge scientific core facilities. Visit us at targetals.org.