

# EVERYONE LIVES

TARGET ALS NEWSLETTER | VOL. 2 | FALL 2023

SIGNALING  
THE DAWN OF  
A NEW ERA:  
2023 ANNUAL  
MEETING

SEVEN PILLAR  
STRATEGY  
TO END ALS

DAN DOCTOROFF:  
CLOSING STRONG







Target ALS core scientific facilities worldwide ALS community. Importantly, this is also an opportunity for investigators to tell us what else they need to accelerate their efforts, and to provide

our support of the community.

The May 2023 Target ALS Annual Meeting was

held in person at the University of California, San Diego. Over 830 attendees joined us in person and online.

Our meeting was a success due to the diversity and expansion of our constituency. There

were representatives from 15 countries and 40 different institutions.





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 analyses of blood samples to non-invasive digital tools  
 to assess speech and gait.



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**W**e continue to exploit the talent and technologies that are part of the ALS research community as an approach to related neurodegenerative disorders that share overlapping genetic causes and disease

and can feature the same underlying pathological

gregation of TDP-43 (a protein critical for maintaining proper production and function of RNA molecules in cells).

molecules that facilitate detection and visualization of very small molecules at high resolution). **This multi-disciplinary**

involves Drs. Jenna Gregory,

Tartaglia and Elsa Zacco, Italian Institute of Technology. This group has developed a technology to detect TDP-43 aggregates

are currently working to adapt this technology to detect

diagnosis and treatment.



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**W**...ative consortia that provides a framework for pharma/biotech-based scientists to lead programs that

... strengths of each constituency: a deep understanding of

This year, we have funded six industry-led collabo-

apeutics, Prosetta Biosciences and Maze Therapeutics as

focused on different approaches to treat ALS that include

proteins called autophagy and C9orf72 form of ALS.

includes Drs. Leonard Petrucelli, Mayo Clinic, Jacksonville,

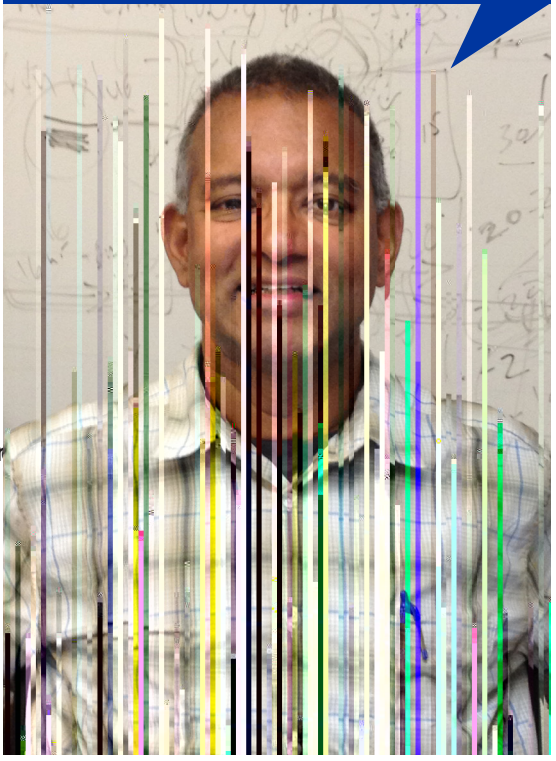
cisco are working together to address the cause of the

board from people with ALS. This group is g... i... i... k g

**TDP-43 aggregation.** Prosetta, a small start-up from the San Francisco Bay Area, has already shown that some of

aggregation in stem cell models of ALS.

“  
We are grateful to Target ALS for providing a mechanism for our novel hypotheses on ALS pathogenesis  
CEO and CTO of Prosetta Biosciences



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nfn o ib g g i g g t i d d d m d m d m  
o n A i i gh i i )A pivotal devel- dm d d m  
opment this year ocured when the FDA Advisory Committee  
voted unanimously that strong reductions in plasma levels of

## PILLAR

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**T**he n-Lorem Foundation is committed to supporting research for all forms of ALS, including the **ultra-rare** disease-causing RNA. Excitingly, recent clinical trials have shown reversal of symptoms following treatment with ASOs in people with ALS who carry

ALS on page 3). We have therefore partnered with

the n-Lorem Foundation to leverage its expertise in ASO technology to expand therapeutic development for ultra-rare forms of ALS. In 2022, we funded clinical studies which allowed for treatment of an ALS patient with an ultra-rare form of mutation in the C9orf72 gene. This event represents a significant milestone in our ASO technology platform. We have now expanded our funding to support the generation of a second ASO therapeutic targeting a distinct ultra-rare ALS mutation.

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## Target ALS Capacity

**W** of our initiatives. This requires that we expand all aspects of our organization to ensure

critical factor in the success of our approach is the role

which **g h i** . This committee consists of **experts from different scientific disciplines,**

**O** **N i i** for their own research, removing any **a d**

We continue to expand the IRC to engage experts in emerging technologies and related neurodegenerative disorders. We are proud to introduce one of the new

cian-scientist and Assistant Professor in the Departments of Neurology, Neuroscience, and Pathology at the Icahn School of Medicine at Mount Sinai in New York City. Her work focuses on understanding vascular aging, examining

dementia due to neurodegenerative diseases.

Target ALS is doing everything right  
ed with ALS or at risk of developing

try researchers together and funded  
multi-disciplinary teams including







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