Tern Therapeutics Receives US FDA Regenerative Medicine Advanced Therapy Designation for Gene Therapy for the Treatment of the Ocular Manifestations of CLN2 Disease

- *Recognizes potential of TTX-381 to address significant unmet need and enables increased dialogue with FDA throughout the development process*
- *RMAT designation granted following FDA review of initial clinical safety and efficacy data from the ongoing Phase 1/2 study of TTX-381 in CLN2 disease, a form of Batten disease*

WASHINGTON, DC, June 3, 2025 — Tern Therapeutics, LLC ("Tern"), a biotechnology company developing transformative one-time gene therapies for rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to TTX-381. TTX-381 is a novel one-time gene therapy product being developed for the treatment of the ocular manifestations of CLN2 disease, a form of Batten disease.

"This RMAT designation represents another meaningful milestone for Tern and underscores the momentum and strength of our TTX-381 program," said Alex Bailey, Ph.D., Chief Executive Officer of Tern. "We look forward to continued engagement with the FDA as we work to accelerate the development of this promising program."

Tern recently reported interim clinical data from the first six participants in its ongoing Phase 1/2 study of TTX-381. Results demonstrated a favorable safety profile, sustained and dose-dependent increases in TPP1 transgene expression, stabilized or improved photoreceptor integrity in all treated eyes, and early signals of stabilized or improved functional vision. These data formed the basis of Tern's RMAT application to the FDA.

"RMAT designation provides strong validation of our approach to treating the rapid vision loss experienced by children with CLN2 and an opportunity to move even more quickly toward fulfilling our commitment to the CLN2 community," said Christina Ohnsman, M.D., Chief Medical Officer of Tern.

RMAT designation is granted by the FDA to regenerative medicine therapies being developed to treat, modify, reverse, or cure serious or life-threatening diseases or conditions. To qualify, therapies must demonstrate preliminary clinical evidence of the potential to address the unmet medical needs for such diseases. Investigational therapies that receive RMAT designation are eligible to receive intensive guidance from the FDA as

well as other actions to expedite development and review, including discussions about potential surrogate or intermediate endpoints and pathways to accelerated approval.

TTX-381 previously received Fast Track, Rare Pediatric Disease, and Orphan Drug Designations from the FDA.

About TTX-381

TTX-381 is an investigational one-time AAV gene therapy designed to deliver a working copy of the *TPP1* gene directly to the retina, potentially providing a durable source of TPP1 to maintain the health of the retina and address vision loss in people with CLN2 disease. Vision loss in children with CLN2 disease rapidly progresses to blindness; there is currently no available treatment for the ocular manifestations of CLN2 disease. The Phase 1/2 clinical trial to evaluate TTX-381 is active and recruiting patients. Learn more at clinicaltrials.gov (NCT05791864).

ABOUT TERN THERAPEUTICS

TERN THERAPEUTICS is a privately held biotechnology company founded in 2023 with a new vision for speeding the development of transformative, one-time gene therapy medicines for rare diseases. Guided by a team of leading physicians, scientists, and business leaders and in collaboration with patient communities, we are driven to deliver transformative treatments with urgency to those living around the world with rare diseases. For more information about Tern, please visit WWW.TERNTX.COM.

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