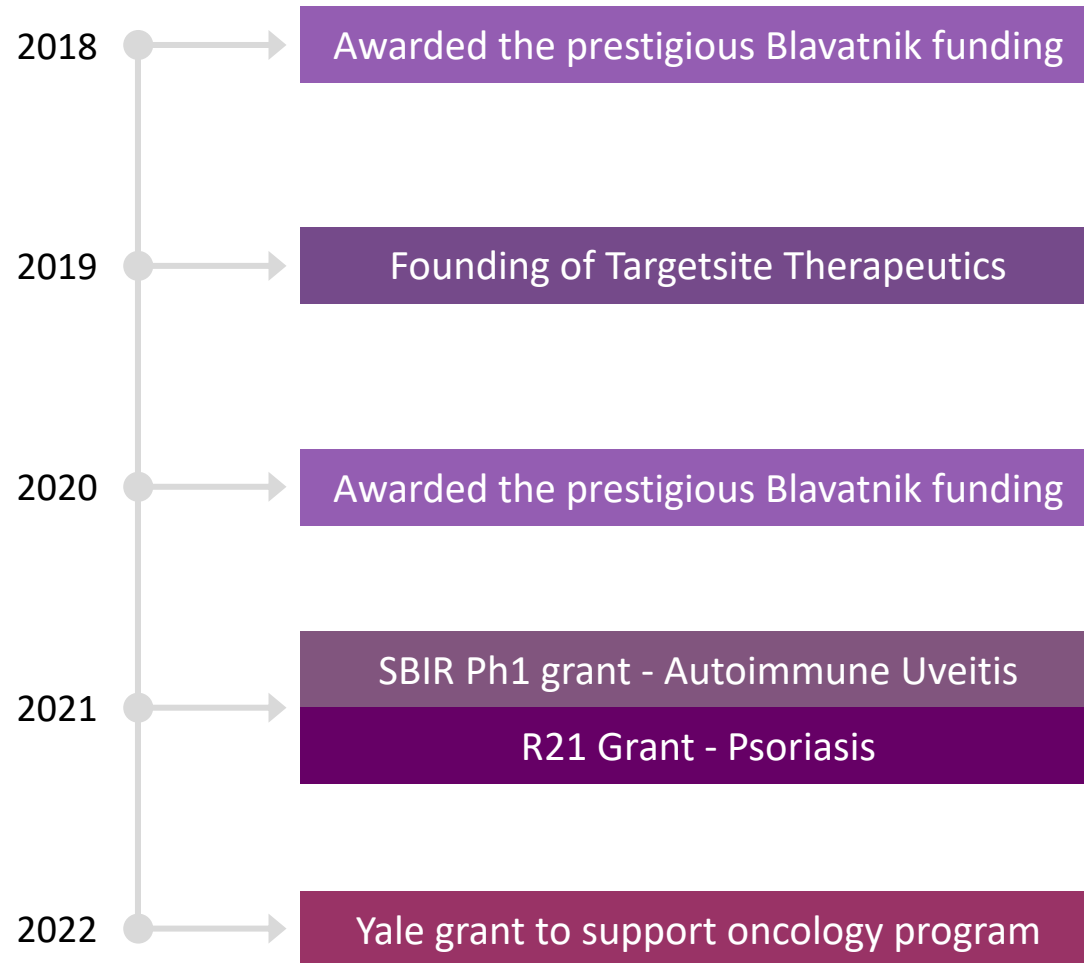


# TargetSite Therapeutics

Harnessing the power of novel mRNA-targeted oligonucleotide therapeutics

# Overview



TargetSite is a Yale spinout, founded in 2019

Building an mRNA-targeted therapeutics platform company, founded on unique insights into mRNA-stabilizing miRNA

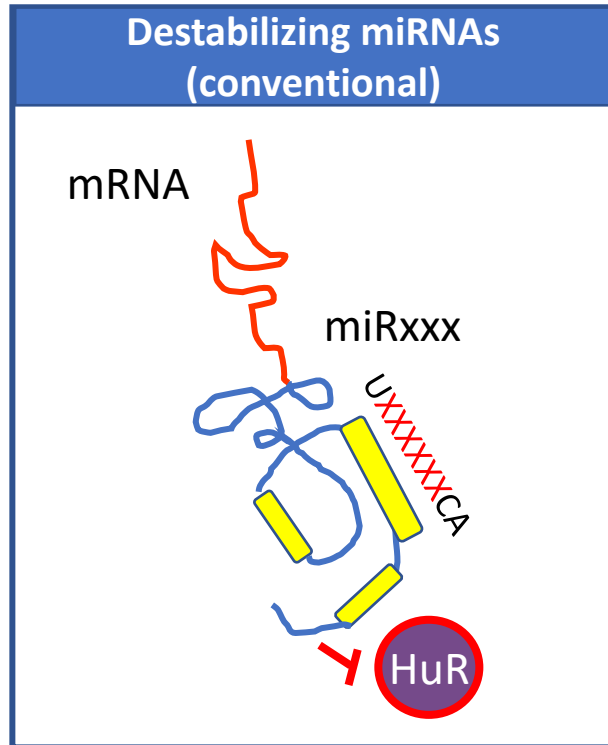
Lead candidate partially de-risked in animal disease models of multiple sclerosis, psoriasis and autoimmune uveitis

Optimization for oral delivery formulations for lead asset in animal diseases models for different autoimmune and inflammatory diseases

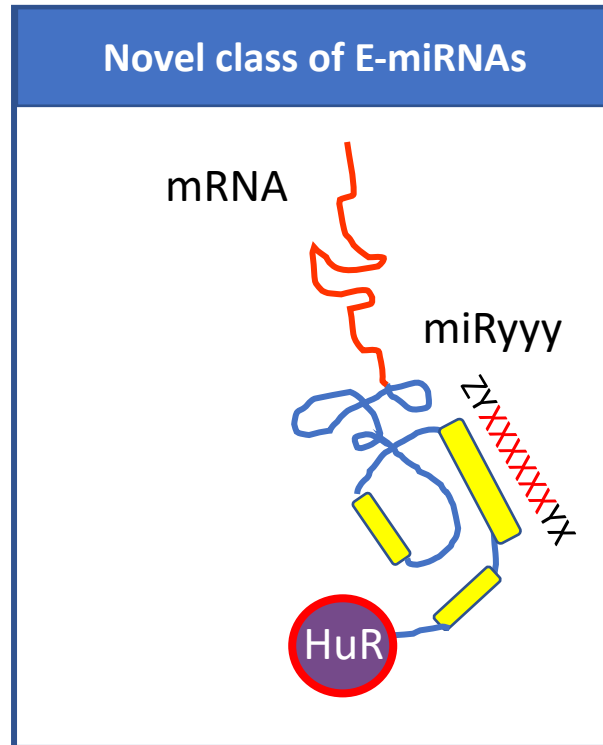
Strong IP position

Looking to raise capital & build strategic partnerships

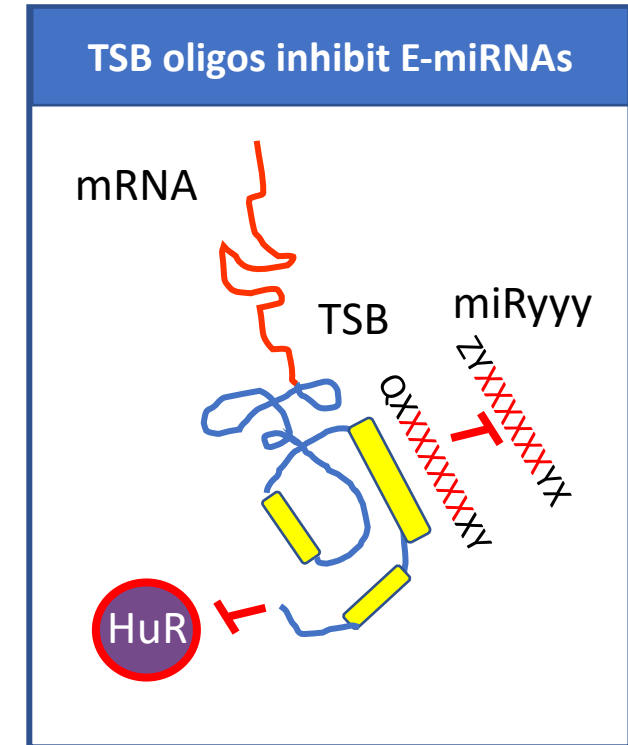
# TargetSite Blockers (TSBs) block Enhancing microRNA's (E-miRNA) interaction with target mRNA



mRNA decay and/or translation block



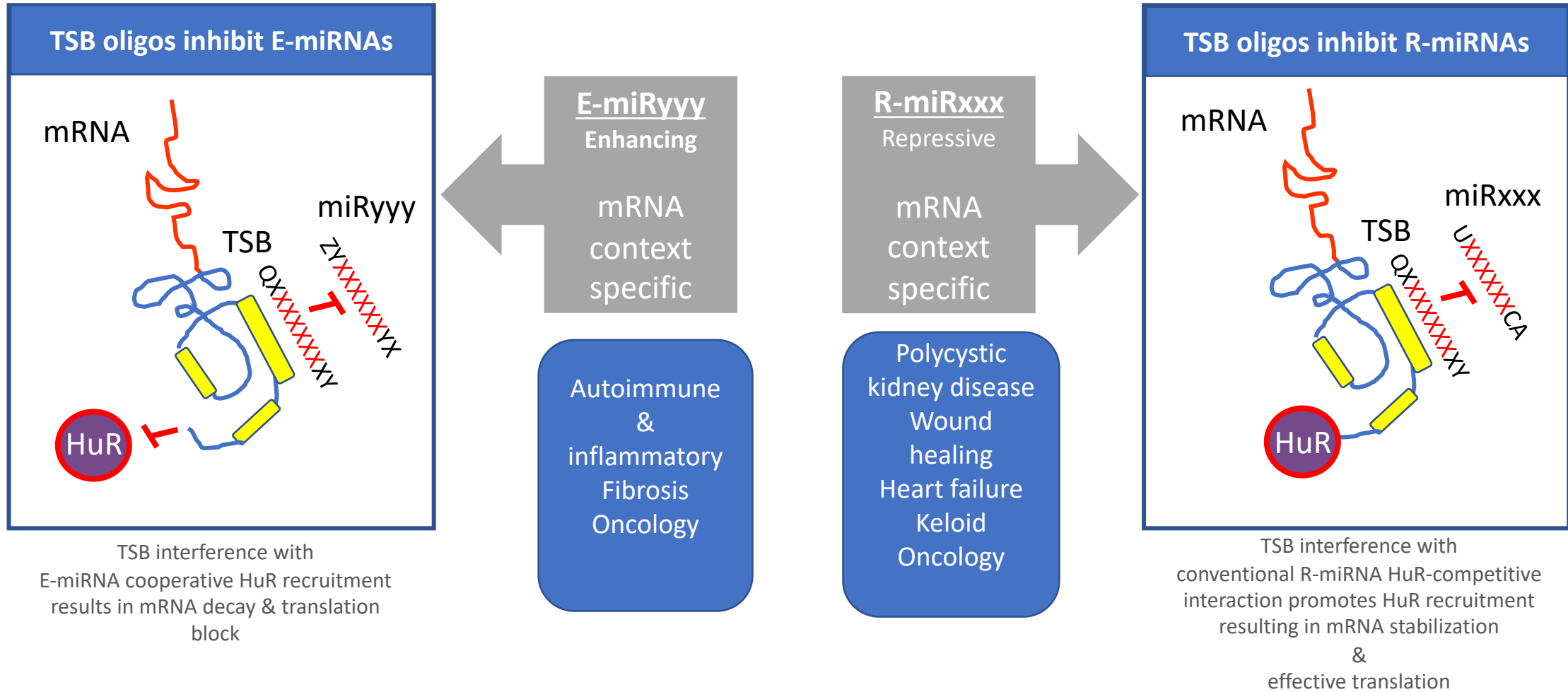
HuR-miRyyy cooperative mRNA stabilization and/or effective translation



TSB interference with E-miRNA-HuR interaction resulting in mRNA decay

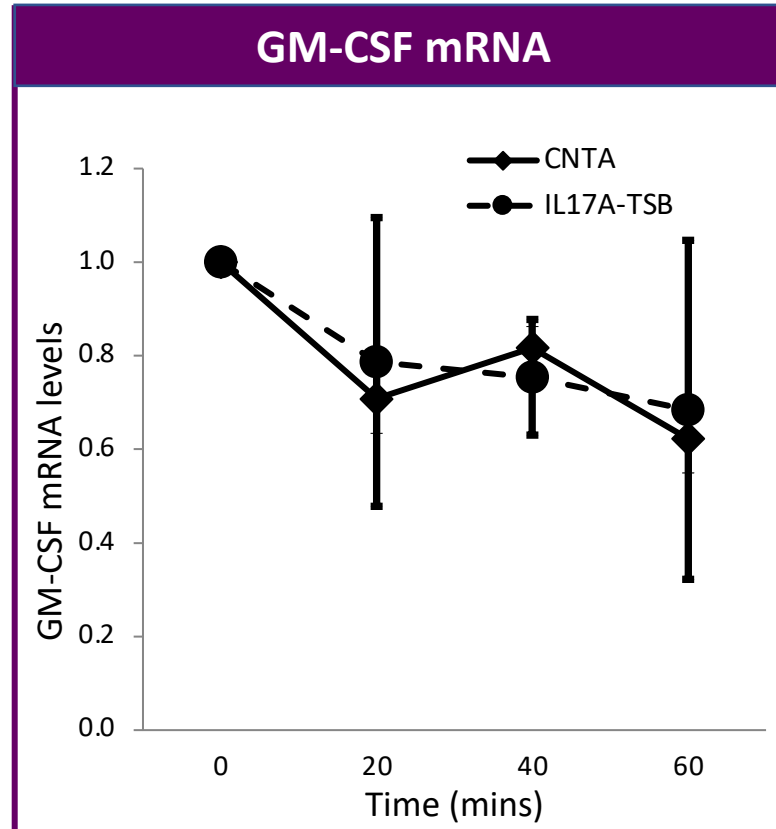
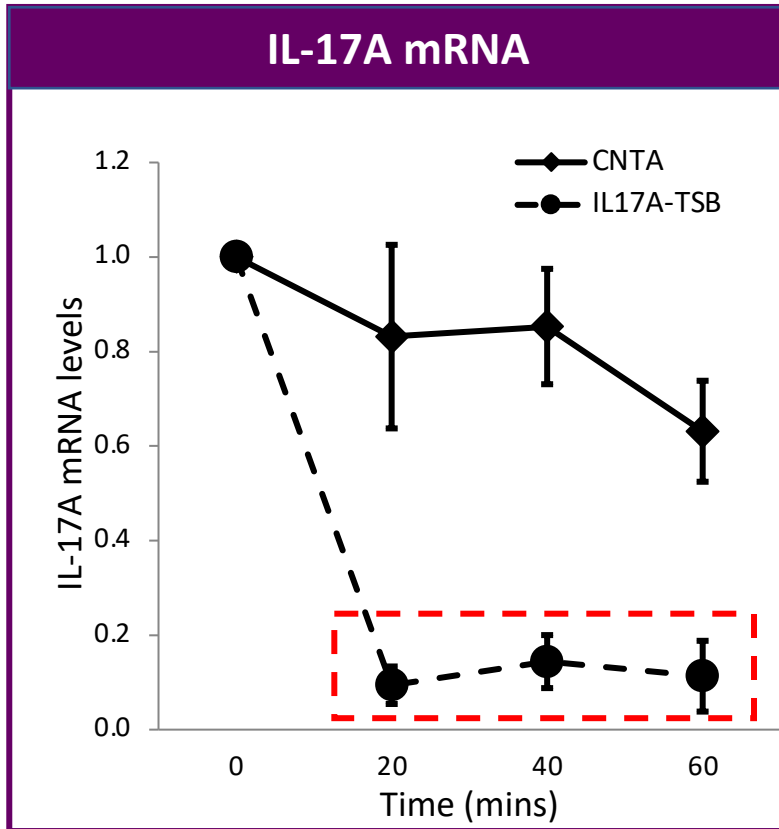
**Blocking the cooperative, translation-promoting HuR-miRNA-3'UTR interaction with sequence-specific modified oligonucleotides can greatly and selectively dampen gene expression**

# TargetSite Blockers (TSBs) for inhibiting & stabilizing target mRNA: Two pillars



Gene expression enhancement or repression dependent on precise nature of miRNA influence on HuR recruitment and binding to any given mRNA 3'UTR

# IL-17A-miRyyy TSB specifically destabilizes human IL-17 mRNA



- Graph showing IL-17A and GM-CSF mRNA decay in human primary T cells
- T cells transfected with 25 nM TSB or CNTA (control) oligo after transcriptional arrest (time 0)

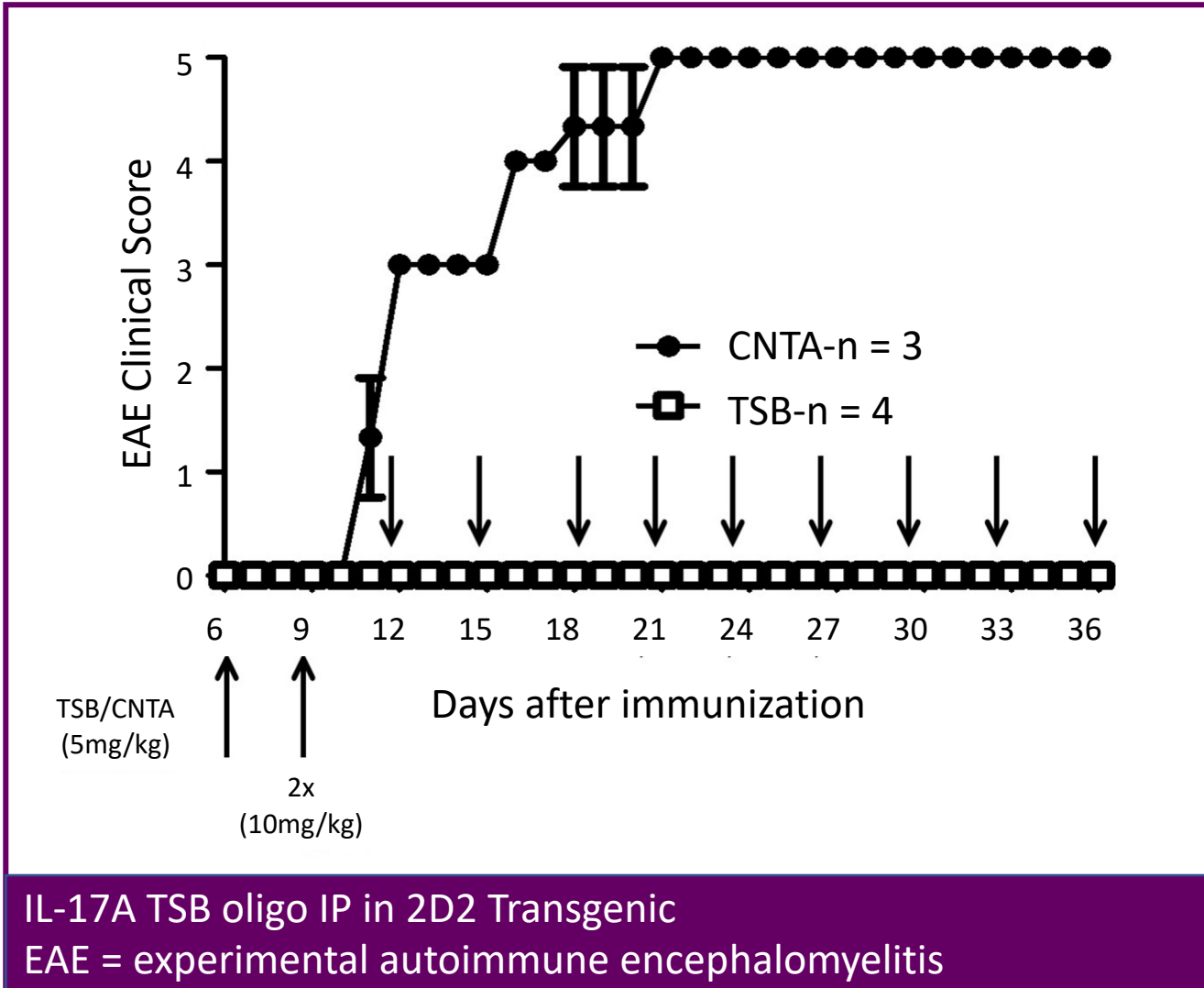
miRyyyy-IL17A TSB prevents HuR-dependent protection of IL-17A but not GM-CSF mRNA

CNTA = Control Oligo A (non-specific oligo)

IL-17A-TSB = IL-17A Target site blocker

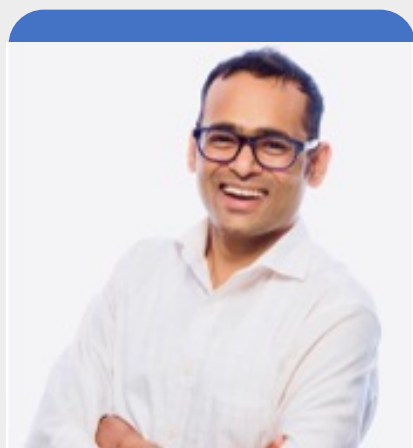
GM-CSF = granulocyte- macrophage colony-stimulating factor

# Complete inhibition of disease in a progressive mouse MS model



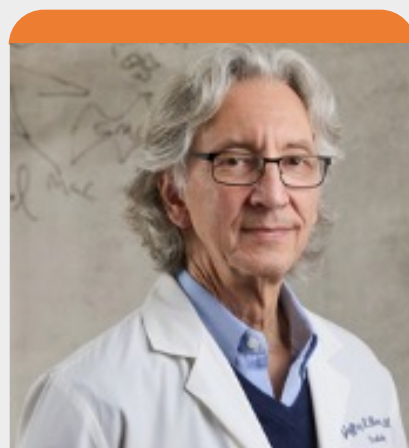
TSB has **high therapeutic potential**

# Team



**CEO & Co-founder**

**Ashoka Madduri, PhD, MBA**



**Co-founder & SAB Chair**

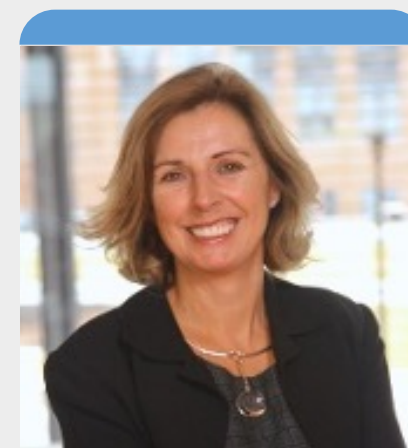
**Jeffrey Bender, MD**

Professor of Cardiology  
Professor of Immunobiology  
Director of the Cardiovascular  
Research Center, Yale



**Co-founder & Head of Discovery**

**Vinod Ramgolam, PhD**



**BD & Licensing**

**Morag Grassie, PhD**

