TargetSite Therapeutics

Harnessing the power of novel mRNA-targeted oligonucleotide therapeutics
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

<table>
<thead>
<tr>
<th>Year</th>
<th>Event</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>2018</td>
<td>Awarded the prestigious Blavatnik funding</td>
<td></td>
</tr>
<tr>
<td>2019</td>
<td>Founding of TargetSite Therapeutics</td>
<td></td>
</tr>
<tr>
<td>2018</td>
<td>Awarded the prestigious Blavatnik funding</td>
<td></td>
</tr>
<tr>
<td>2020</td>
<td>SBIR Ph1 grant - Autoimmune Uveitis</td>
<td>R21 Grant - Psoriasis</td>
</tr>
<tr>
<td>2021</td>
<td>Yale grant to support oncology program</td>
<td></td>
</tr>
</tbody>
</table>
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)

miRyyy-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

IL-17A TSB oligo IP in 2D2 Transgenic
EAE = experimental autoimmune encephalomyelitis
EAE score is a score of MS disease severity
Near-term research plan

Optimize TSB oligos oral delivery formulation in collaboration with Matinas BioPharma Holdings oral deliver technology company to target immune & inflammatory cells: ongoing collaboration with a company with expertise in oral delivery formulation

Advance lead oral asset into IND enabling stage

Disease mouse model studies for oral delivery (efficacy & tox): MS, IPF, Dry AMD, IBD

Advance the platform
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