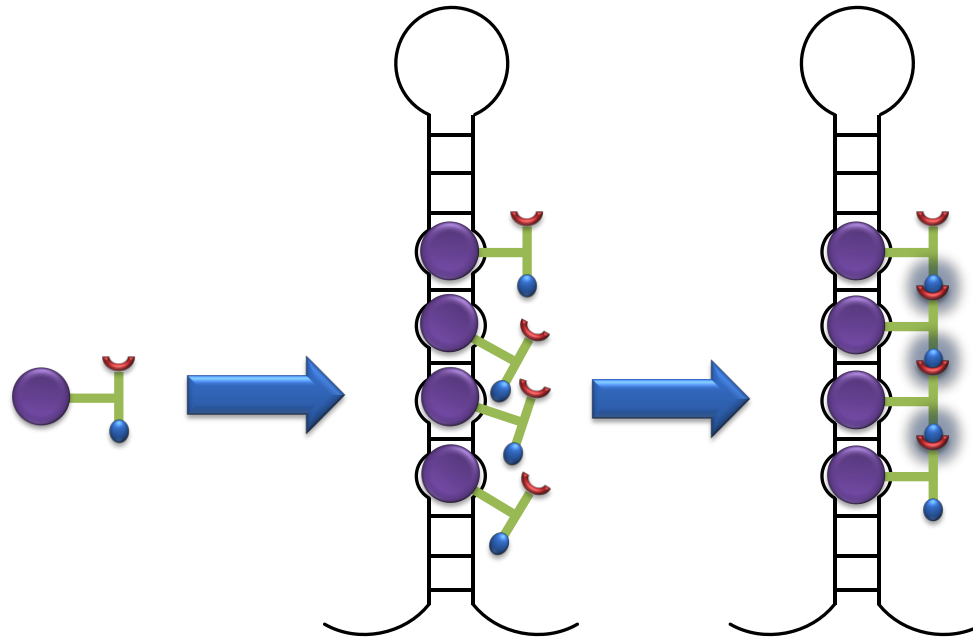


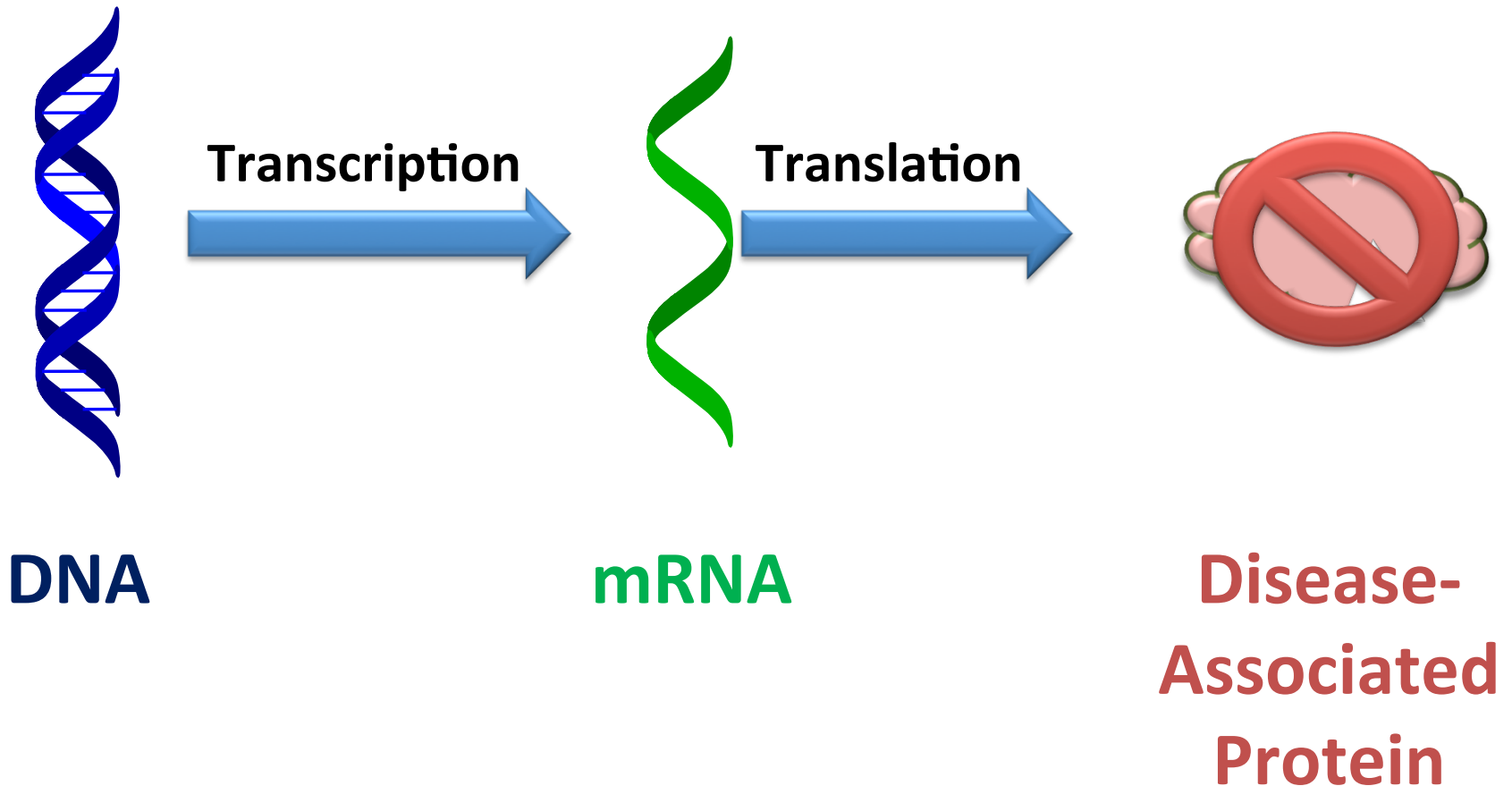
Using a Toxic RNA to Synthesize Its Own Inhibitor



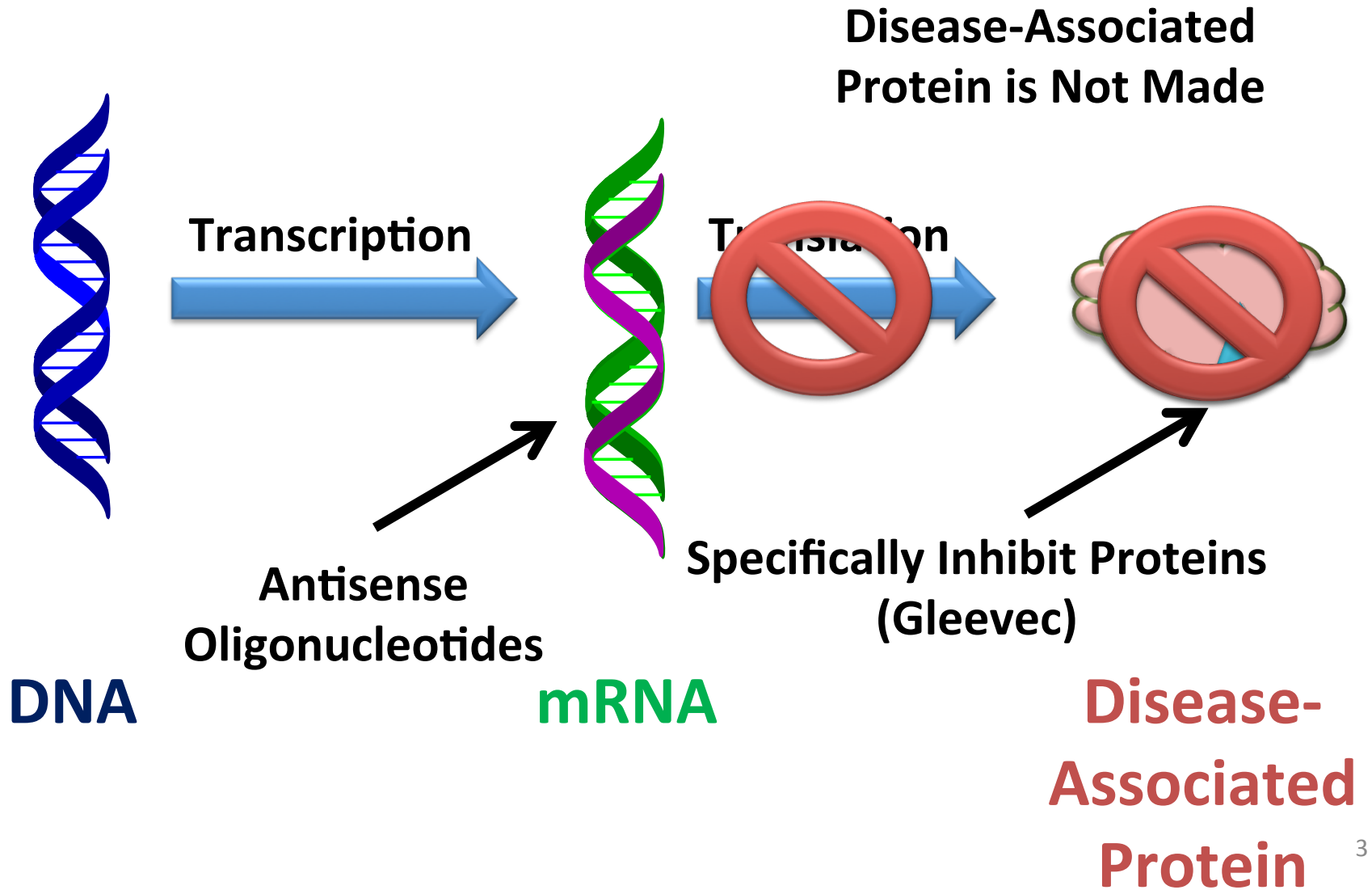
Suzanne Rzuczek Ph.D.
Disney Lab
Department of Chemistry
The Scripps Research Institute
September 12, 2014

Precise Medicines

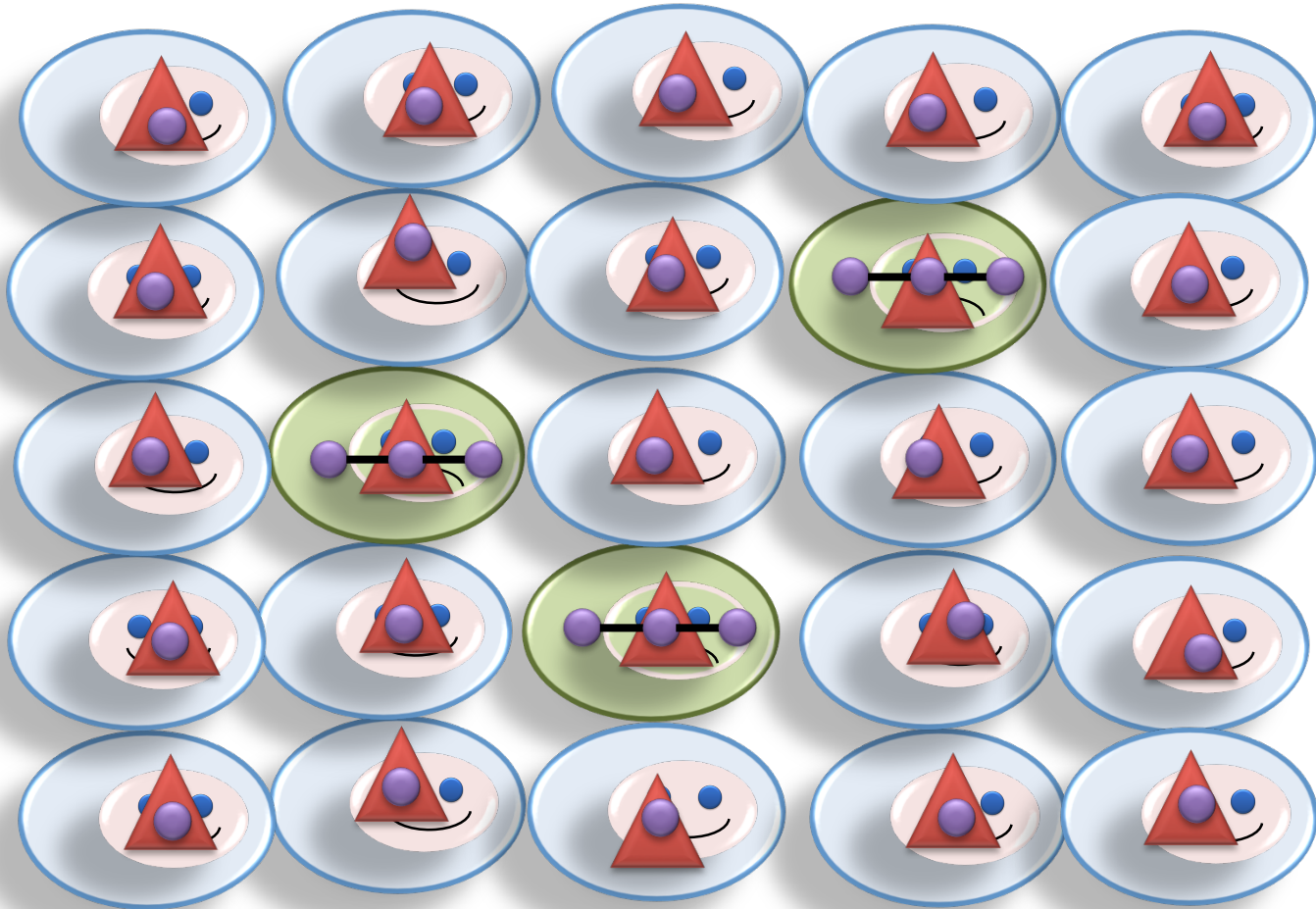
Create Target-Specific Therapies



Examples of Precise Medicines

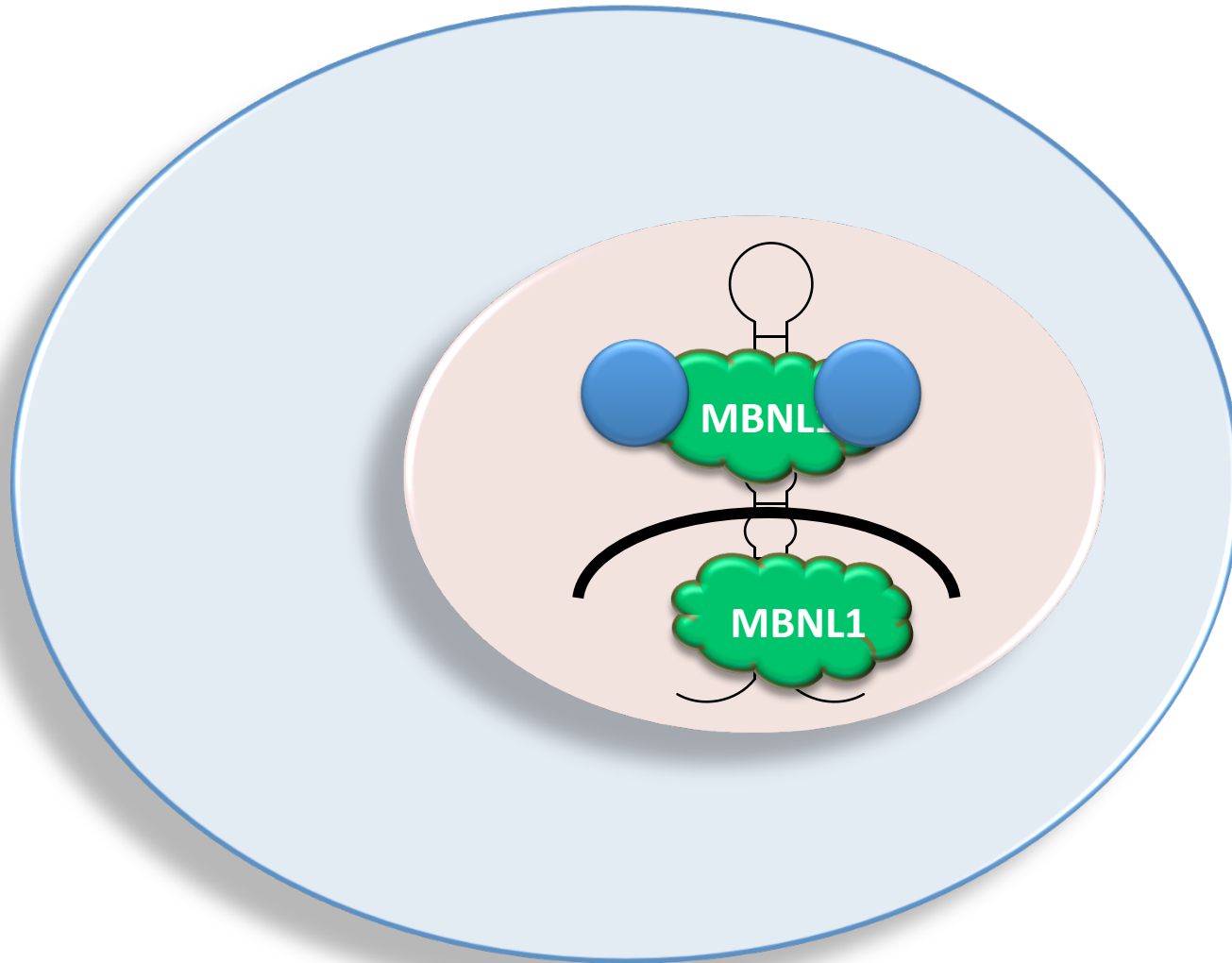


Optimizing Precise Medicines

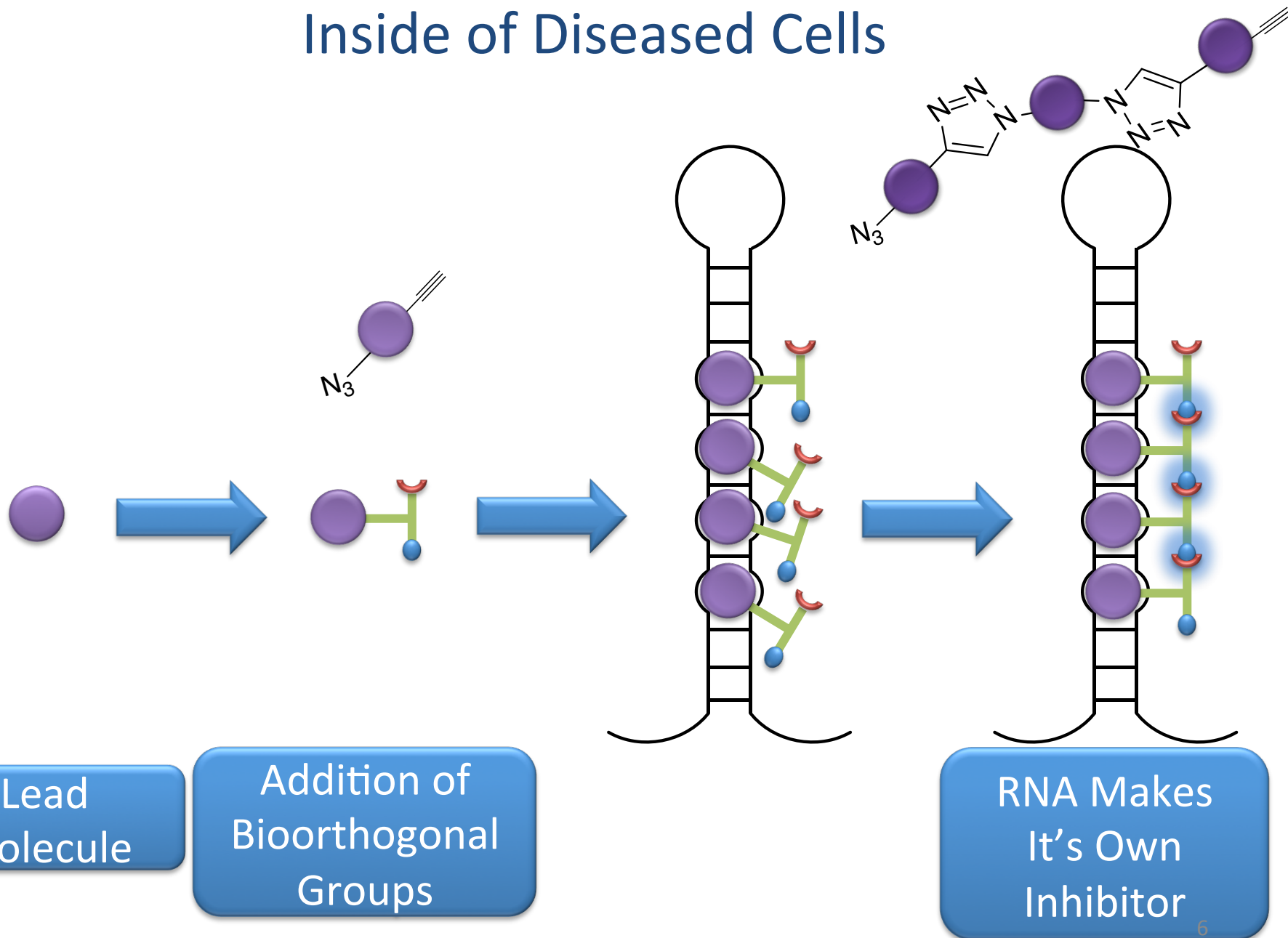


Healthy and Diseased Cells are Exposed to the Same Amount of Treatment

Cellular Characteristics of DM



Techniques for Synthesizing Potent Molecules Inside of Diseased Cells



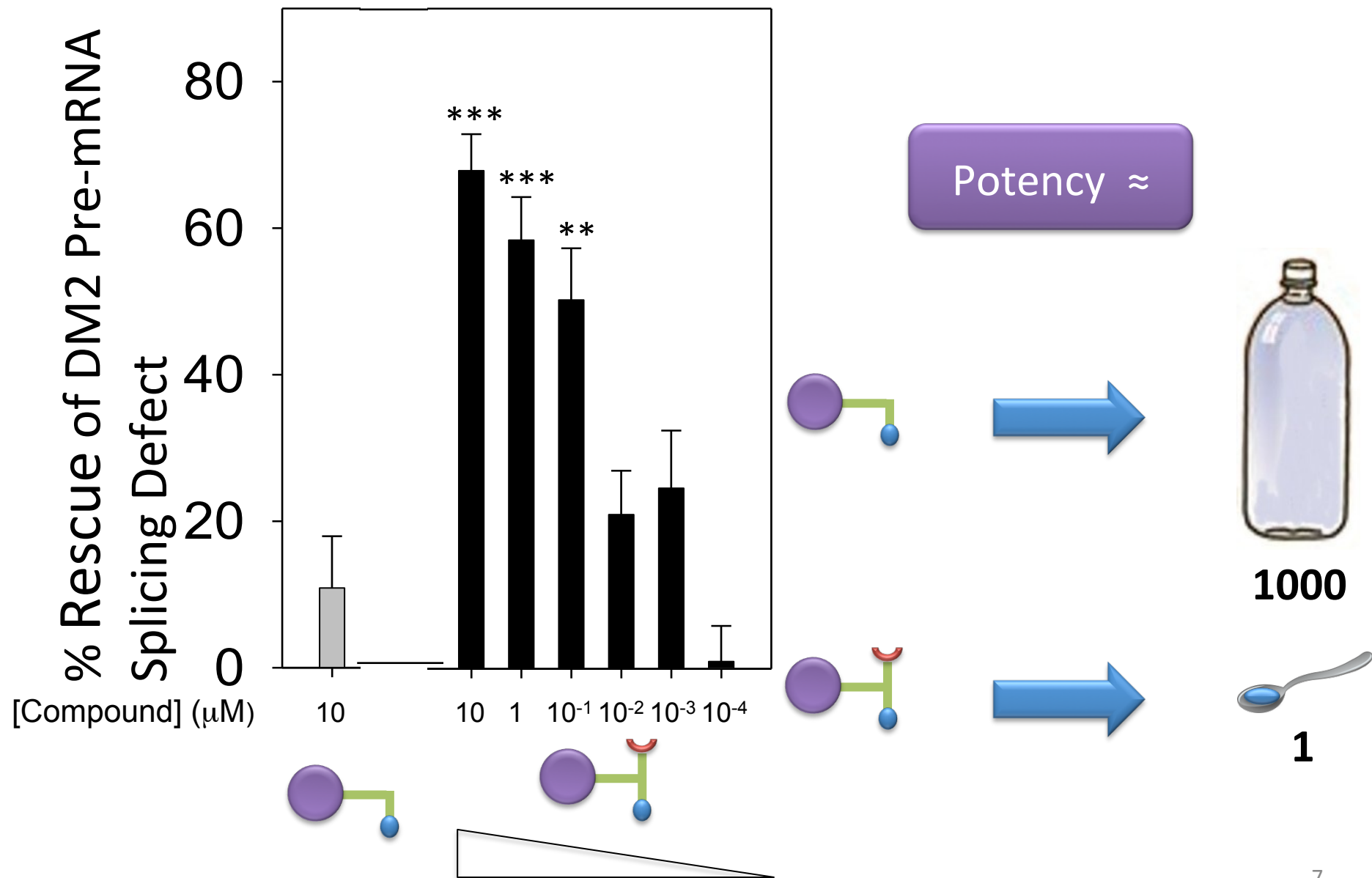
Lead
Molecule

Addition of
Bioorthogonal
Groups

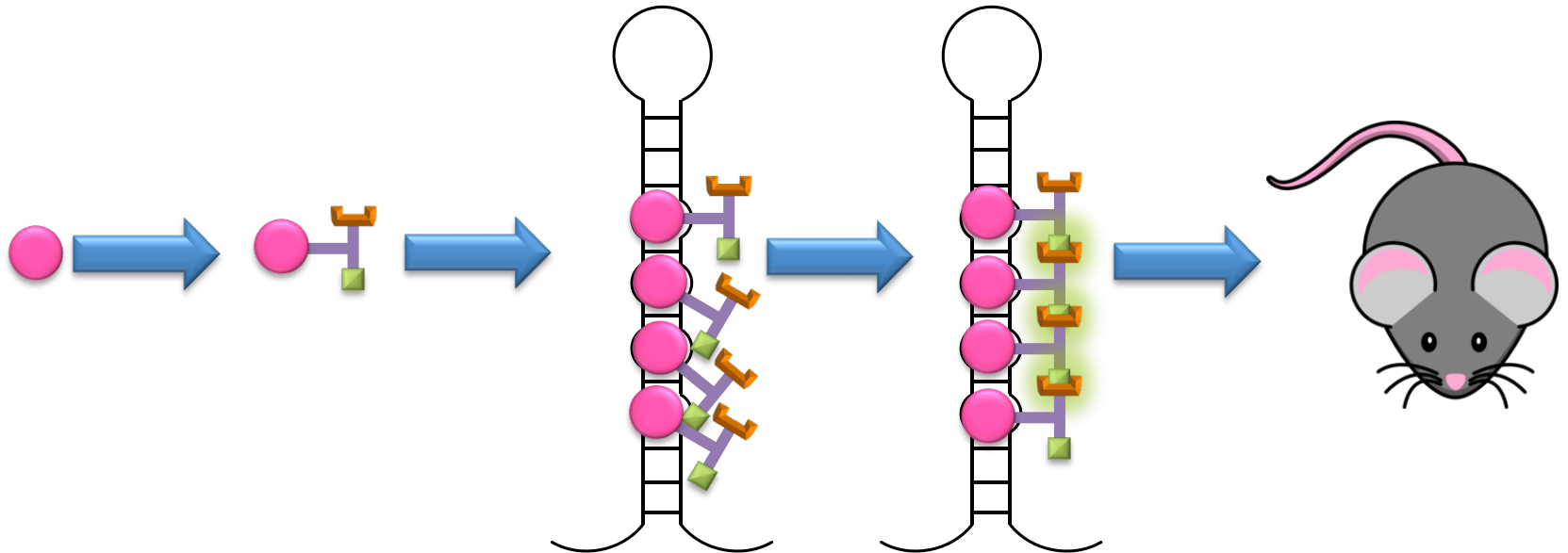
RNA Makes
It's Own
Inhibitor

6

Inhibitors Synthesized by RNA Targets Are Highly Potent



Future Studies



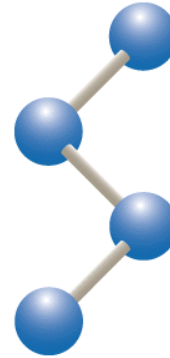
Develop this technology for DM1

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MYOTONIC
DYSTROPHY
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