

# Drug Discovery/Pipeline

## Translational Research and the Drug Discovery Process

### Translational Research

Translational research is the name given to the continuous process of moving the knowledge gained from basic early stage “bench” research through various stages of the drug discovery process. Early stage basic research leads to groundbreaking discoveries that set the stage for development of therapeutics to treat disease. In early stage basic research, investigators identify new targets that can then be interrogated by small molecule compounds (drugs), biologicals (e.g. antibodies or vaccines) or gene targeting compounds (gene therapy). To move from an initially identified target to the development and testing of new therapeutics takes many steps.

### Path through the drug discovery “pipeline”

Traditionally, teams of medicinal chemists, pharmacologists and biologists screen many thousands of compounds to screen for “lead compounds.” These molecules have certain desirable properties, but researchers usually must modify them to increase their activity or minimize side effects - a process called “lead optimization”. Following this process, hundreds of potential drugs are generated for pre-clinical testing.

### Drug Discovery & Development Cost

According to the Pharmaceutical Research and Manufacturers of America (PhRMA), it takes 10-15 years to develop a single drug at an average cost of \$1.3- \$1.7B. For every 5,000 – 10,000 potential drugs that are initially tested in an animal model of disease only 250 will progress to pre-clinical development. Of these 5 will move forward into first-in-man studies (Phase I) of which only a single compound will survive to be an approved drug.

### A new paradigm in Research Funding

Given the immense cost, long time-frame and high failure rates, it is absolutely critical to provide a rational means of feeding new drugs into the development pipeline in order to increase the overall number of “shots on goal” and increase the likelihood of finding a disease-modifying therapy to reverse Rett syndrome.

## Addressing the “Translation Gap” in early stage drug discovery

Following a careful review of research funding over the past 10 years, funding of Rett syndrome research (IRSF Landscape Analysis), a critical gap was identified which exists in translating basic research and early stage drug development. As part of our new funding strategy, IRSF will seek to bridge this “Translation Gap”

IRSF leadership recently conducted a strategic planning process to begin to specifically target funding that will move translational research forward and bridge the gap. Once potential drug targets have been identified and properly validated it is necessary to follow these leads with continued funding to push the most promising programs through the pipeline until they are ultimately tested in clinical trials.