Orphan Drug Development Guidebook

Milestone: Target and Product Discovery

Typically, researchers discover new drugs through:

- New insights into a disease process that allow researchers to design a product to stop or reverse the effects of the disease.
- Many tests of molecular compounds to find possible beneficial effects against any of a large number of diseases.
- Existing treatments that have unanticipated effects.
- New technologies, such as those that provide new ways to target medical products to specific sites within the body or to manipulate genetic material.

To select the best drug, researchers engage in an often long phase of work to deepen the understanding of the molecular/ cellular mechanisms to be targeted and its impact on the disease processes, as well as building virtual, cellular, and animal models that reliably reproduce the role of such mechanism. This phase is called Target Validation and enables researchers and developers to create the basic knowledge and tools that will be needed inn the following phases.

At this stage in the process, thousands of compounds may be potential candidates for development as a medical treatment. After early testing, however, only a small number of compounds look promising and call for further study.

Once researchers identify a promising compound for development, they conduct experiments to gather information on:

- How it is absorbed, distributed, metabolized, and excreted.
- Its potential benefits and mechanisms of action.
- The best dosage.
- The best way to give the drug (such as by mouth or injection).
- Side effects or adverse events that can often be referred to as toxicity.
- How it affects different groups of people (such as by gender, race, or ethnicity) differently.
- How it interacts with other drugs and treatments.
- Its effectiveness as compared with similar drugs.

This fishing and selection phase is called Drug Discovery: its scope is to produce a single (or limited number) of best candidate products responding to all the characteristics that can make them (in theory and experimentally in the lab) a suitable drug for patients.