

DEVELOPING PREVENTIVE INTERVENTIONS AND THERAPEUTICS



Target validation.

Potential therapeutic targets identified in discovery research are confirmed to play a causative role in a given disease.



Target to hit.

Large numbers of chemical or biological agents are screened to identify molecules that “hit” the target.



Hit to lead.

Positive hits are further tested to determine which bind the target with the most specificity.



Lead optimization.

The properties of the lead compound are refined to enhance potency and drug availability and to reduce side effects.



Preclinical testing.

Cellular and animal models are used to test for effectiveness of the optimized lead, identify any potential toxicity issues, and determine an optimal starting dose for clinical or “first-in-human” testing. The final compound is called the clinical candidate.

IND

Investigational new drug.

Prior to clinical testing, one or more clinical candidates are submitted to the FDA for approval to be used in clinical trials.

5K-10K
COMPOUNDS

5-10 YEARS

1-5



Adapted from (1)