NASCIC
SCI RESEARCH
ADVOCACY COURSE

Module 2 – Understanding the Research Process and R&D Decision Making

Roles for Advocates in Drug R&D

Research advocates can improve every stage of drug R&D, but it's best to start at the beginning because of the way R&D is planned: most drug sponsors sketch out the whole process before they start based on the label they want the drug to have.

A drug label is a long and detailed document that describes the drug, the specific health conditions and people it is approved for, the approved doses, the risks associated with its use, and--many pages later--its efficacy in clinical trials. It is an important document, because a sponsor cannot make any marketing claim about how well the drug works, how safe it is, who it is for, or how it should be used unless that claim is included in the drug's label.

The label is approved by regulators when they approve a drug.

Because of that, drug development starts by establishing goals for what the label should say. This set of goals is often written down in a document called a target product profile, or TPP. The TPP includes what condition or symptoms the drug should treat, which patients should use it, how it should be dosed, how much efficacy it should have, and what side effects or risks are acceptable.

The sponsor works backward from these goals to design clinical studies that will result in the desired label. Each phase of the development process is planned out so that it will provide the information needed to take the next step.

What this means is that by the time a drug sponsor begins R&D, they have made decisions about what the drug should do and how it should be developed based on the desired drug label.

That is why advocates should be engaged in research from the very beginning.

In discovery and preclinical development, advocates help shape the product profile by providing information on unmet needs, the burdens of their health condition, and the burdens of existing treatments, if there are any. Advocates can also shape the product profile by providing data from their community about the expectations of benefit and the tolerance for risk. They can advise on product characteristics, administration, and packaging. And, importantly, they can help assess the relevance of early research to the community's needs.

During clinical development, advocates can advise sponsors on eligibility requirements and other barriers to participation. They can help design participant materials, identify and prioritize what studies measure, assist with recruiting volunteers, and provide feedback on the participant experience.

Advocates also play important roles in the approval of new drugs, by commenting on regulations and guidance documents, testifying at public hearings about drug development, and serving on advisory committees.