NASCIC
SCI RESEARCH
ADVOCACY COURSE

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

Exceptions and Special Circumstances

The R&D processes we've just described are used for the vast majority of drugs and medical devices, but there are some exceptions and special circumstances when these products can be approved with less data.

Let's start with drugs. The most common exception to the typical development pathway is when a sponsor seeks a new approval for a drug that is already approved to treat a different condition. In these cases, clinical testing can usually start in Phase 2. There are similar exceptions for new formulations of an existing drug, such as developing a pill form of a drug that is already approved as an injection.

Regulators also make exceptions for drugs intended to treat serious conditions that have no approved alternatives. In the U.S., this is called Accelerated Approval. In Europe, it's called Conditional Approval.

Under these pathways, drugs can be approved after only Phase I or Phase II studies if the studies show the drug is safe and reasonably likely to be effective. The sponsor is required to confirm efficacy in larger studies after Accelerated or Conditional approval, and if those studies fail, approval can be revoked.

Drugs that are developed for rare health conditions may be eligible for Orphan Drug designation. Rare means that not many people have the health condition. In most countries, that means less than 200,000 people. In the U.S., FDA considers SCI to be an Orphan indication.

Orphan drugs can often be approved with smaller and fewer trials than other drugs because there are fewer eligible people, and those people are harder to find and enroll in studies. Approval still requires evidence of safety and efficacy, but all three phases of clinical development may not be required.

Similarly, a medical device intended to treat fewer than 8,000 people may be eligible for approval in the U.S. without evidence of efficacy under the Humanitarian Device Exemption. The sponsor must show that there is no other way that the device could be brought to market, and that there is no comparable device already available.

In the U.S., clinical studies of drugs or medical devices that are intended for use in emergency settings also may be eligible for a waiver from informed consent. An IRB may grant this kind of waiver only when treatment must be provided quickly, patients are incapacitated, and a legally

authorized representative is not readily available to provide consent. In these cases, before the study starts, FDA requires sponsors to consult with representatives of the communities in which the trial will be conducted, as well as public disclosure to these communities.

These waivers are rare, but are of interest in SCI because of the difficulty obtaining informed consent when an SCI has first happened. We will talk more about this topic, and other challenges in SCI research, in Module 3: Addressing Historical Challenges for Research Studies.