NASCIC
SCI RESEARCH
ADVOCACY COURSE

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

Basic and Translational Research

Before a medicine or a medical device can be developed, scientists in universities, government, and medical research centers have spent years doing experiments to answer fundamental questions about how injuries and illness cause damage, and how and why our bodies respond to that damage. This part of the process is called basic and translational research, and is essential to understanding how a treatment might be able to stop or reverse the effects of damage.

We call the first step "basic" research because it answers basic questions about why and how something works. Translational research builds upon that fundamental knowledge to figure out how to stop, slow, or reverse damage from disease or injury. Translational research can also uncover new questions for basic research to answer.

Basic and translational science experiments are conducted in laboratory tests, called "assays," that use cells grown in the lab or samples of blood or tissue from people who have the condition being studied, and in animal models that have characteristics that closely resemble the human health condition being studied.

Examples of questions that basic and translational research experiments in SCI try to answer include:

- What cells and molecules are involved in the body's response to an SCI?
- How do these cells and molecules respond to an SCI?
- Why do these cells and molecules behave the way they do?
- Can we change the way these cells and molecules behave so that damage can be stopped or repaired?

Here's an example of a real-life basic research experiment that has led to several drug development programs.

Scientists noticed that damaged nerves outside the brain and spinal cord, called "peripheral nerves," can regenerate themselves after injury, but damaged nerves in the brain and spinal cord, which make up the "central nervous system," or "CNS" do not.

The research question was: Why?

Basic researchers answered part of this question in the 1980s by doing experiments in rodents. The experiments showed that when injured nerves from the CNS were provided with a "bridge" made from a peripheral nerve, they could grow and be directed toward a specific area.

Separate experiment showed the reverse: when injured peripheral nerve cells were provided with CNS tissue as a bridge, the peripheral nerves failed to grow.

Together, these experiments showed that both peripheral and CNS nerves have the potential to grow back after injury, but something in their physical environment must be determining whether or not they are able to do so.

Since then, researchers have gone on to identify several of the key cells and molecules that inhibit regrowth in the CNS and promote it in the peripheral nervous system. These cells and molecules are now targets for drug development programs in clinical testing. We will talk more about these molecules, and the drug programs that are targeting them, in Module 9.

Basic and translational research are usually conducted by researchers in universities, government, and medical research centers.

The factors that influence what research gets done are very different than they are for drug and device R&D. Individual scientists want to do research that is scientifically interesting and "novel," meaning that the research will break new ground. They are incentivized to do research that has the potential to be published in prestigious scientific or medical journals, and that might result in patents, because publications and patents are how scientists further their careers. And, of course, the research that they actually get to do is influenced by the availability of funding.

This type of research is paid for by a variety of public and private funders. The largest funder of basic and translational research is the U.S. National Institutes of Health, or NIH, which has an annual budget in the tens of billions of dollars.

Other funders of basic and translational research include large private foundations such as the Bill & Melinda Gates Foundation, non-profit scientific institutions such as the Howard Hughes Medical Institute, and organizations that focus on specific health conditions such as The Craig H. Nielsen Foundation, The Christopher and Dana Reeves Foundation, and The Praxis Spinal Cord Institute.

Most of the funding for basic and translational research is distributed through competitive grant programs, in which researchers apply for the money to pay for specific projects. Grant applications are reviewed and scored by other scientists, and the funding institution uses these reviews to decide what to fund.

The review criteria vary depending on the institution's goals. Common criteria include:

- Scientific interest, or novelty;

- The potential to help understand or solve a medical problem, especially for organizations that are focused on a specific health condition;
- How feasible the research project is; and
- The track record of the researcher who is requesting funds.

Research advocates have an extremely important role to play in basic and translational research: they bring a real-world perspective to what scientific questions are most relevant to their community.

Sometimes, research advocates can see opportunities that researchers might not think of. That's because people who live with a medical condition know better than anyone else what medical problems need to be understood and solved.

Research advocates can also help think through how basic and translational research might be applied, during R&D or in healthcare, and identify gaps in information or problems that can limit future usefulness.

In other words, research advocates can help ensure that basic and translational research is not only interesting, but also useful.