Translational medicine research at Novartis

Too often, new therapies fail to bridge the treacherous gap that lies between research and entry into human clinical testing. Our team of translational medicine researchers work to bridge this gap and ensure that safe, effective and innovative treatments reach patients as quickly as possible.

When NASH develops in the human liver, fat builds up (white) and collagen fibers accumulate (pink), resulting in scarring that interferes with the liver’s ability to function. Image by Chandra Saravanan/Novartis

Led by Evan Beckman, the group applies its medical and research expertise to guide promising therapeutic candidates along the path to development. Our physician-scientists have a deep understanding of disease biology and unmet medical needs. Working shoulder-to-shoulder with our discovery colleagues, and alongside a dedicated team of preclinical safety, pharmacokinetic, biomarker and clinical scientists, we identify and validate new drug targets, compounds and biological drugs, and provide insights on where novel therapies can achieve the greatest clinical benefits.

Before a new therapy is given to people for the first time, scientists in Translational Medicine first rigorously test our drugs in preclinical studies in animal models for safety and pharmacokinetic properties. Many of these scientific experts from Translational Medicine remain with the therapy as it moves into the clinic and into patients throughout the drug development process.

Our group also develops biomarkers and assays to investigate whether a given therapy alters the same disease pathways in people as it does in preclinical studies in model organisms. By using imaging data, genetic expression profiles and other powerful tools, we are able to explore treatment responses in far more sophisticated ways than was possible in the past. In that way, we let our science point us towards the best medical applications for a new therapy, instead of committing to just one disease area early on.

We evaluate experimental therapies by:

- **Characterizing their properties** and performing initial tests to confirm they act on specified targets and are safe for people;
- **Designing and conducting focused studies** in small numbers of patients to understand a candidate’s therapeutic potential in different diseases; and
- **Identifying the diseases** that a therapeutic candidate will likely treat most effectively

These small-scale studies are typically carried out in smaller homogeneous populations of patients within a disease and sometimes in rare genetic diseases. The results of this careful initial testing help us streamline future efforts in diseases that may be more widespread. For instance, based on responses to an experimental therapy in patients with rare blood and kidney disorders, such as paroxysmal nocturnal hemoglobinuria and C3 glomerulopathy, we pivoted towards testing it in patients with more common conditions including IgA nephropathy, and membranous nephropathy. (Learn about a patient’s experience with IgA nephropathy [here](#).)

“We’re taking great ideas in early research and finding suitable opportunities to advance them toward the next phases in development,” Beckman says. “And in doing that, we’re thinking hard about the biology and science of human diseases, while also applying a clinician's judgment to guide our therapies to the places they might have the biggest impact and change the practice of medicine.”
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