

## FINDING, FILLING, AND FUNDING GAPS IN DIABETES RESEARCH

As the field of diabetes research has grown over the years, JDRF's role within that industry has evolved dramatically. Some 35 years ago, the organization was a pioneer, creating awareness of the need for research leading to a cure. Over the next 25 years, JDRF shaped the direction of scientific investigation as a primary funder and partner with academia and others in exploratory and basic research. And in the past few years, we have moved increasingly to a portfolio of managed research: science aimed at delivering cures and therapeutics to market, with greater involvement with industry participants, strategic projects, drug discovery, and human clinical trials.

Today, more than ever before, JDRF's research focus is characterized by an ability to understand where there are gaps in research, and to then move quickly to fill them. Filling gaps has always been an important aspect of the organization's role, whether it was the gap in focusing on diabetes as an area of scientific investigation in 1970, or the level of involvement of NIH in diabetes research in 1999. But the progress and growth of diabetes and complications research to its current state have created a new range of gaps – from financial and integration-related, to strategic or advocacy-related. JDRF's research strategy moving forward is aimed at identifying and plugging those gaps that would otherwise prevent research from moving as quickly – if at all.

### Funding Gap

The most common gap is a funding gap. As more and more research moves beyond exploratory science, aimed at testing concepts, but not yet at the stage where industry can begin the process of developing specific products and treatments, a significant gap has emerged in how translational research is financed. Venture capital funding is less widely available than in the past, and industry is less likely to commit resources at that stage of the product pipeline. In the coming years, much of JDRF's research portfolio will be focused on moving science from that exploratory phase into the product pipeline. Already, much of our industry funding supports programs that otherwise would not have advanced to the stage they have without JDRF involvement.

### Integrational Gaps

Similarly, a series of integrational gaps are emerging, as it becomes more clear that developing cure therapeutics for a disease as complex as type 1 diabetes will require combined solutions – for example, turning off the autoimmune response that causes diabetes, while simultaneously spurring beta cells to regenerate. Both industry and academic research tend to be organized around disciplines: small molecules vs. biologics vs. cell based therapies in the case of industry, or immunology vs. cell biology vs. developmental biology in the case of academia. JDRF's overview of diabetes research puts the organization in a perfect position to fill that role, and we are already leading integrated multi-discipline, team-science research efforts in regeneration and immunity.



## Scientific Gaps

Scientific gaps can occur for several reasons. Academic exploratory research can result in advances that might hold promise for diabetes cures and therapeutics – but the translation of that science moves into areas beyond the scope or focus of the original investigators. Similarly, researchers focusing on other diseases or disciplines might uncover compounds or biological pathways that suggest opportunities for diabetes therapeutics. JDRF’s high-throughput screening of existing drugs and compounds approved for use with other diseases is a good example of how the organization can address gaps in science.

## Proficiency Gaps

As products and treatments come to market, JDRF is filling proficiency gaps, ranging from shepherding concepts through the regulatory approval process to helping gain clinical acceptance and patient usage. Again, JDRF’s position as a leader in diabetes research provides both a level of experience and a degree of gravitas that may accelerate the introduction of cure therapeutics to a wide range of people with diabetes. The organization’s ongoing work with mechanical interventions, such as continuous glucose monitors and the development of an artificial pancreas, are clear examples of unique JDRF proficiencies complementing scientific and product engineering expertise.

## Organizational Gaps

And there are ever-emerging organizational gaps that JDRF is in a position to fill. Product development is rarely a straight-line event in industry. Research can be overlooked, slowed, or even derailed because of organizational priorities, resource allocation, or product timetables. JDRF is able to fill gaps created by conflicting organizational priorities by providing a platform for research that has been shifted to a back-burner, exploring alternative targets for drug development, or changing the economic dynamics of therapeutics development. That can turn projects that held only marginal interest for a drug company into priorities. Transition Therapeutics’ clinical trials involving the regenerative agents GLP-1 analogs and gastrin are a good example, with JDRF stepping in to provide both sponsorship and funding when Transition Therapeutics’ former biotech partner chose to focus its resources on other compound development.