

Imagine a disease. It steals your child's sight. It has no cure. It is so rare that it is not understood by the medical community. It is unclear what it will do next.

Jeff and Cathy Sawler have experienced the pain and isolation of such a disease with their daughter, Tarah.

Tarah was born with the genetic condition known as FEVR, which creates varying degrees of visual impairment. By the age of eleven, FEVR had completely robbed Tarah of vision.

"When I think of what I've gone through," says the bright, active and musically-talented 13-year-old, "It's just not right that someone else would have to go through it."

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The IGNITE project is taking an innovative approach to FEVR and other rare disorders to find new information and discoveries that can lead to prevention, treatments and cures for people like Tarah and her family.

**IGNITE**  
SPARKING HOPE FOR  
ORPHAN DISEASES

Learn more about Tarah and other families involved in the research project at [www.igniteproject.ca](http://www.igniteproject.ca)

#### PARTNERSHIPS

Collaborations are the cornerstone of the IGNITE project's success. Sharing information, expertise and effort enables faster and more readily-validated results.

Our expertise can bring us to the brink of pharmaceutical development by establishing the genetic links behind orphan diseases, the potential targets for therapeutic intervention, and the compounds best suited for pharmaceutical development.

**But we need strategic,  
forward-thinking commercial partners  
to help us take it to the next level.**

Specifically, we are looking for partners who can optimize promising compounds, conduct clinical trials and collaborate on other important aspects of the drug development process.

We are also seeking to add more compounds to our screening process, which will increase the odds of finding therapeutics for specific diseases. If you have compound libraries that you are interested in sharing for the study of one or more diseases, we would be happy to collaborate.

Some orphan diseases can inform the treatment of common, high-impact disorders such as cancer, diabetes and hypertension. The IGNITE project's unique and extensive experience enables us to provide partnering opportunities to groups who are interested in the development of diagnostics and/or drug treatments for common diseases that share features with much rarer entities.

If you have a specific disease, condition or phenotype under evaluation, we would be happy to hear from you, and to determine if there is a way we can use our platform to collaborate. In turn, we are always looking for partners to help us fulfill our mandate of finding the genetic basis and potential treatment for the orphan diseases listed on our website.

#### ACKNOWLEDGEMENTS

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For more information on the project or partnering opportunities, please visit our website, or contact us.

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Rare or 'orphan diseases' are so named because they are often neglected by the research and pharmaceutical communities. But, sadly, orphan diseases collectively impact 1 in 12 Canadians.

## Orphan diseases impact 1 in 12 Canadians.

That's roughly 3 million men, women and children who live with a disorder that has relatively few opportunities for effective treatment or improvement.

The IGNITE project aims to bring hope to these people through the use of state of the art scientific research that will lead to more informed and effective care.

[www.igniteproject.ca](http://www.igniteproject.ca)

### THE PROJECT

## One project. Many results.

The IGNITE project is focused on helping those with orphan diseases. It is designed to address the issue from a variety of angles, using cutting-edge technology and world-class approaches.

THE PROJECT HAS FIVE CLEAR GOALS:

1. Discover the genes responsible for orphan diseases in Atlantic Canada and elsewhere
2. Pinpoint targets most likely to respond to therapeutics
3. Identify drugs to treat these targets
4. Generate intellectual property for commercial licensing partnering
5. Develop an integrated understanding of the important social and policy issues surrounding orphan diseases

## Powerful tools. Powerful outcomes.

Genomics is a combination of biology, genetics and computer science. It harnesses the power of all three of these disciplines to enable us to discover and better understand our genes and how they impact our development, health and illness.

The IGNITE project is using genomics to find the genes behind these rare diseases. When we find the gene, we are then able to develop highly accurate diagnostic tools to screen patients for the disease. A faster, more reliable diagnosis can lead to more effective treatment, and more efficient use of patient and healthcare time and resources.

## Small targets. Big rewards.

One of the activities of the IGNITE project involves finding the molecular pathways that show the most potential to respond to drug treatments. With this information, we can then find the drugs to treat them.

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## Old drugs. New uses.

The commercial drug development process can take years and hundreds of millions of dollars, making it difficult for pharmaceutical companies to justify investing in drugs that can only be used by small numbers of patients. However, many pharmaceutical companies are now interested in repurposing existing, approved drugs for different diseases.

The IGNITE project is leveraging this interest by focusing on drugs and small molecules that have a desired impact on the molecular pathways impacted by orphan diseases. By identifying these drugs, and then testing them on other organisms, they hope to determine which existing drugs may provide effective treatment for these rare diseases. As these drugs and molecules have already passed the first phase of clinical trials, the cost and time associated with developing them into approved and commercially available drugs will be significantly reduced.

## Human disease. Human issues.

The IGNITE project addresses the ethical, economic, environmental, legal and social aspects of the genomics research.

The project will look at regulations around orphan drug funding, attitudes to genetic testing for these disorders, and appropriate methods to provide test results.

### TEAM

The IGNITE project team is a diverse and talented group of leaders with strategic experience in orphan drug research and development. The team's internationally-recognized combination of expertise and global networks uniquely positions them to produce fast, actionable results to bring hope to those with orphan diseases.

THE TEAM IS CO-LED BY:

**Dr. Christopher McMaster,**  
Professor of Pediatrics and Biochemistry & Molecular Biology,  
Assistant Dean of Medicine, Dalhousie University

**Conrad Fernandez, MD**  
Professor of Pediatric Hematology/ Oncology in the Department  
of Pediatrics, Dalhousie University/IWK Health Centre

For more information on partnering with the IGNITE team, please see the reverse.