CFA
CANADIAN FABRY ASSOCIATION

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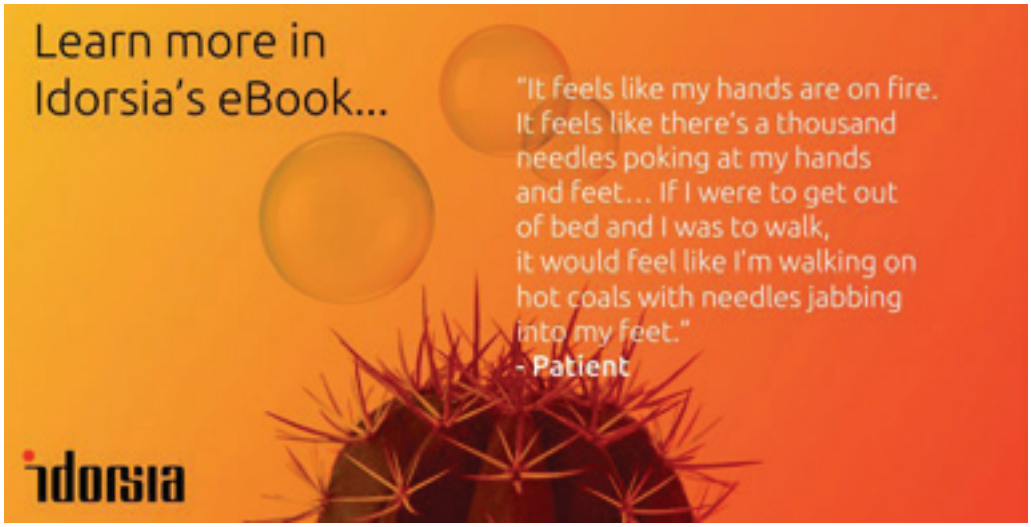
NEWSLETTER



IDORSIA PHARMACEUTICALS ANNOUNCEMENT

Idorsia Pharmaceuticals (www.idorsia.com), a Swiss biotech company, is developing **lucerastat**, a new oral therapy for patients with Fabry Disease. The Phase 3 clinical study MODIFY has just enrolled its first patients and will investigate the use of lucerastat monotherapy for the treatment of adult patients with Fabry disease, irrespective of their genetic mutation type.

The MODIFY study will enrol patients having Fabry neuropathic pain despite long-term use of enzyme replacement therapy (ERT) and willing to discontinue ERT for the duration of the study, as well as patients having Fabry neuropathic pain while not receiving ERT. Following a screening period of 6-7 weeks, patients enrolled into MODIFY will receive lucerastat treatment or placebo for six months before being offered the opportunity to enter an open label extension study. The primary endpoint of the study is a reduction in neuropathic pain, which Fabry patients report as significantly impacting their daily activities and quality of life, despite existing treatments.

If you are an adult patient with Fabry disease and have neuropathic pain, and you are interested in participating, please contact your doctor or contact a clinical site near you.

More information on MODIFY including a list of clinical trial sites where the study will take place can be found on: https://www.clinicaltrials.gov/ct2/show/NCT03425539?term=lucerastat&rank=3

Julia Alton
Executive Director

PATIENT EMPOWERMENT QUESTION:

How many Fabry mutations have been identified.

SURVEY: PATIENTS LIVING WITH FABRY DISEASE AND THEIR CAREGIVERS

The Canadian Fabry Association (CFA) is conducting a short survey with patients living with Fabry disease and their caregivers.

If you qualify and successfully complete the survey, you will have the option to enter in a draw to win a CAN $150 gift certificate to Keg Steakhouse.

You may complete the survey only once.

Your opinion is essential to helping the CFA best meet the needs of the Canadian Fabry community. Please click here (https://www.surveymonkey.com/r/Fabry2018) to make your voice heard.

Thank you so much in advance!

Julia Alton
Executive Director

BE RARE. BE YOU.

Tattoo

I was asked to write a little something about why i got our "Be Rare Be You" fake tattoos actually tattooed on me.

Well that's easy. Since i am into tattoos and I'm often asked about them and i thought this would be a great way to bring more awareness about Fabry.

I have my own painting business and I always tell my customers about Fabry just in case i have a bad day and can't work or I have my infusion. Everyone is always understanding and usually asked more about it.

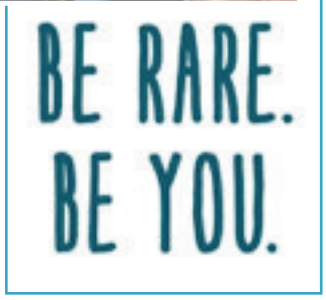
And now i have people asking me about my new tattoo. So far it is working as planned.

I look forward to spreading more awarness about our rare disease.

"BE RARE BE YOU"

Matt Lock
CFA Member

PATIENT EMPOWERMENT CALGARY MEETING - COMMENTS



"Having other patient interaction and meeting so many brave individuals and understanding their journey."

"This was my first meeting and definitely not my last. Excellent presentations and flow of the day - I have a much better understanding of my recent diagnosis."

"Great combination of scientific information with practical life based applications."

PATIENT EMPOWERMENT ANSWER:

A. Over 900 mutations!

THANKS TO OUR SUPPORTERS

We would like to thank all of our supporters that helped make this newsletter possible.

We receive financial support from thee Pharmaceutical companies who are currently providing hope for Fabry patients through their research and the products they provide.

Amicus Therapeutics

genzyme

Shire

PROTALIX Biotherapeutics

We would also like to thank all of the physicians, specialists and medical professionals that have helped in so many ways. From providing guidance on medical terms and details to caring for members of our community every day.

And of course we would like to thank all of the patients and family members that have volunteered their time and energy to assist in all the many ways that are necessary in the creation of such a large effort. It is through their efforts that we hope to inform and build a community of Fabry patients for the benefit of patients, their families and caregivers.

MAKE A DONATION

Would you or a family member like to make a donation so that we can continue to educate and advocate for the best treatment as well as communicating with and for Fabry patients in Canada?

The Canadian Fabry Association (CFA) is a registered not-for-profit organization.
If you are interested in making a charitable donation and would like a tax receipt, please make your cheque payable to The Fabry's Charity Association.

100% of donations to the CFA are used to promote education, patient support and access to treatment for Canadian Fabry patients. You can make donation cheques payable to The Fabry's Charity Association and mail the cheque to us.

Send the cheque to:
The Fabry's Charity Association
748 Kelly Street
Thunder Bay, ON
P7E 2A1
or register online by visiting our website:
www.fabrycanada.com

Thanks for your donation to the CFA! It goes to help Canada Fabry patients, their families and caregivers.