CFA
CANADIAN FABRY ASSOCIATION

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NEWSLETTER

in this issue:

RARE DISEASE DAY 2017

Lee Straus
Board Member

The incredible amount of support demonstrated throughout the CFA's First Virtual Initiative was exceptional. This is a testament to the genuine support available to the Fabry Community.

Thank you to everyone who participated in making this Rare Disease Day a success. The buzz of this campaign created an outlet to share stories, for our friends to ask questions, and for the word "Fabry" to be recognized.

Stronger Together.

TRIVIA QUESTION:

What animal (rodent) cell does Sanofi Genzyme use to make Fabrazyme?

THANKS TO GINA CONSTANTINO

Julia Alton
Executive Director

On behalf of the CFA and Fabry Community we want to thank our former President Gina Constantino for her dedication, energy, and time. Gina worked with passion in her heart, and was a strong advocate and leader. In Gina's tenure, she held our very first Fabulous Female Retreat, held many Regional Meetings and a very successful National Patient Meeting in 2016. Gina is, and will always be an incredible voice for all Fabry patients.

Thank you Gina for leading our Fabry Community and for the strides you have made. We thank you for your dedication and we wish you a bright, healthy, and successful future.

Your Fellow Board Members

JULIA ALTON
NEW EXECUTIVE DIRECTOR

Julia Alton
Executive Director

My name is Julia Alton (former Vice President of the CFA) and I am proud to share that I am taking on the new role as Executive Director. I look forward to working towards a common goal with all of you - to Live Well with Fabry Disease.

Supporting Industry

Research and clinical trials are needed to improve the understanding, management, and treatment of Fabry disease. We need everyone's support to benefit Fabry Patients globally.

We are supporting two important initiatives and a few moments of your time help to make strides in reasearch. In time, we can further support additional initiatives such as Amicus Therapeutic's, and additional potential new therapies by Genzyme, Sangamo, and others.

But for now, please do these important things:

1. Actelion, a leading pharmaceutical company active in orphan diseases, is seeking insights from our patient community to support clinical research with Lucerastat as a possible new therapy for Fabry disease. Lucerastat is an oral drug that has the potential to provide substrate reduction therapy (SRT).

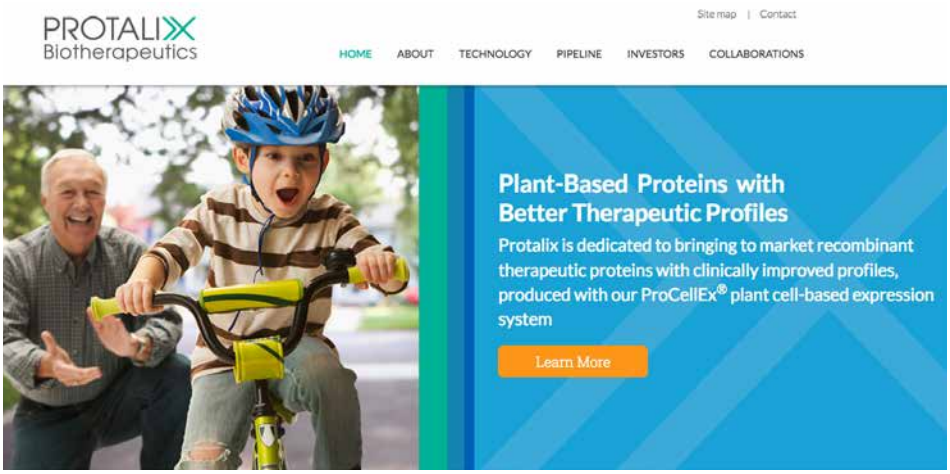
Click on the survey below to help gather research (the survey takes 5 minutes and Canadian participation is needed).
http://interactive.schlesingerassociates.com/survey/selfserve/1819/161111?code=XXXX#?

2. Protalix Biotherapeutics continues to seek additional participants for their two ongoing Phase 3 clinical trials for the investigational drug, PRX-102. If you have not yet inquired about their ongoing clinical trials to determine if participating is right for you, please visit *www.fabrynext.com.*

Please complete the contact information form at the bottom of the *www.fabrynext.com* website page to learn even more about the trials.

Thank you for your time and participation.
Every little bit helps.

PROTALIX UPDATE



Dear Canadian Patients and Families,

Protalix is running a clinical research study, and is looking for patients to volunteer. Protalix has a plant-based Enzyme Replacement Therapy (ERT) and is running two trials:

BALANCE

The BALANCE study is in Phase III of their clinical trial. They are looking for ~ 80 patients who match the following criteria:

1. 8-60 years of age
2 Have worsening kidney function
3. Have been on agalsidase beta (Fabrazyme) for approximately 1 year

There will be two patient groups in this study, two out of every three patients will receive the investigational medication. One out of every three patients will receive agalsidase beta. This study will take place over a two year period.

BRIDGE

The BRIDGE study is in Phase III of their clinical trial as well. They are looking for 22 patients who match the following criteria:

1. 18-60 years of age
2. Experience symptoms of Fabry Disease, such as pain and/or angiokeratoma
3. Have been on agalsidase alpha (Replagal) for at least 2 years.

All patients will continue their current dose of agalsidase alfa for at least 3 months of the study. These 3 months are the screening period. If patients qualify for the study after this screening period they will then receive the investigational infusion.

Taking part of this study is voluntary and patients can leave the study anytime. Protalix is looking for our support, if you qualify or know of any patients that meet the criteria and are interested please contact *patient-info@protalix.com*. You can also find more information at *www.fabrynext.com*.

THE WEBSITE HAS BEEN LAUNCHED!

The Website Committee - Ryan, Lee

Hello everyone, as mentioned in earlier Newsletters, we have been working on a redesign of our website. The progress has been continuing and we are finally complete! The site features dynamic content that can easily be updated to keep everyone as up-to-date as possible. Our new website will conform to the wide variety of devices used to connect to the internet, be it a phone, tablet, desktop or laptop. This will mean less scrolling and faster access to the information you want to see.

Go to *www.fabrycanada.com* to have a look!

ON THE HORIZON

Donna Straus
Board Member

Early plans for a Family Fabry Camp are in the making. Stay tuned for dates and exciting details regarding this incredible weekend.

REMEMBERING FRIENDS

Would you like to have a note of remembrance included in our newsletter? These notices would be for Fabry Patients and Association Members whom we have lost over the years. Please contact us at: secretary@fabrycanada.com

DONATIONS AS MEMORIUM

We have been asked if they can make a donation to the Fabry's Charity Association as a Memorium for their family member. The answer is Yes. Please contact us at: secretary@fabrycanada.com

RECIPE FOR KIDNEY PATIENTS -

PASTA PRIMAVERA

Portions: 6

Serving size: 1-3/4 cup

Ingredients:

- 12 ounces pasta, uncooked
- 12 ounces frozen mixed vegetables
- 14 ounces low-sodium chicken broth
- 2 tablespoons all-purpose white flour
- 1/4 cup half & half creamer
- 1/4 teaspoon garlic powder
- 1/4 cup grated Parmesan cheese

Preparation:

Cook pasta and vegetables in separate pots according to package directions, but omitting salt. Drain.

Pour low-sodium chicken broth in a medium-sized stockpot and heat on low heat.

Add flour to broth whisking vigorously to avoid clumps from forming.
Add half and half and garlic powder and stir.

Simmer on low heat for 5 to 10 minutes until mixture thickens slightly. Stir occasionally while simmering.

Add cooked vegetables and pasta.
Cook until heated through.

Sprinkle with Parmesan cheese and serve.

TRIVIA ANSWER

Making Fabrazyme begins by inserting the human gene for the alpha-GAL enzyme (the enzyme that is deficient in people with Fabry disease) into CHO (Chinese hamster ovary) cells.
The majority of biotechnology drug products produced today are from CHO cells.

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.