CFA
CANADIAN FABRY ASSOCIATION
volume II issue iii
fall 2020

NEWSLETTER

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AVROBIO is a gene therapy company

AVROBIO is a leading clinical-stage gene therapy company driven by a purpose to people with lysosomal disorders from a lifetime of genetic disease. We aim to halt or reverse disease throughout the body by driving durable expression of functional protein, even in hard-to-reach tissues and organs including the brain, muscle and bone.

Learning from the Fabry community

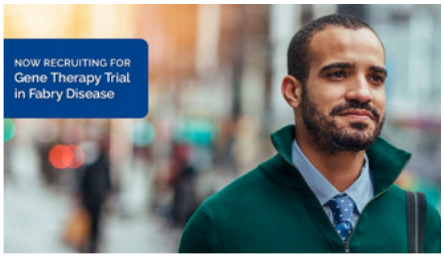
In September 2020, AVROBIO hosted a meeting with leaders of the Fabry patient advocacy community from the U.S., U.K., Netherlands, and Spain to discuss and obtain advice on our investigational gene therapy program for Fabry disease and hear about the needs of the community. We are grateful for the opportunity to speak with and learn from the Fabry patient community!

AVROBIO is recruiting patients for FAB-GT, a phase 1/2 clinical trial (NCT03454893)

AVROBIO is developing an investigational lentiviral gene therapy called AVR-RD-01 for Fabry disease. AVR-RD-01 is being investigated in clinical trials and has not yet been approved by the U.S. Food and Drug Administration (FDA) or any other regulatory agency, and its safety and efficacy have not yet been established.

FAB-GT is a Phase 1/2 clinical trial evaluating the safety, tolerability and efficacy of AVR-RD-01 for the potential treatment of classic Fabry disease. We are currently recruiting patients for this trial. If you or someone you love are interested in learning more about this trial, please contact 1-877-330-5216 or visit www.AVROBIOFabryTrial.com.

Please join AVROBIO at National Fabry Disease Foundation's Fabry Family Conference.



Please take the opportunity to stop by AVROBIO's presentation at NFDF's Fabry Family Conference where we'll provide information about our investigational gene therapy program for Fabry. We also encourage everyone to stop by and say hello in our virtual booth. We hope to see you there!

For more information, please visit AVROBIO.com or reach out to patients@avrobio.com.

Cardiac Month

Julia Alton
Executive Director

I would like to thank everyone for their participation in Cardiac Month! From the heart healthy pictures, webinars, and Zoomba, it's been so wonderful to see everyone get involved!

We were pleased to have Helen Sawyer, Katie Hanley, Marcella Bird, Lori Culum, and one more who prefers to stay anonymous as our 5 winners!

PATIENT EMPOWERMENT QUESTION:

Q. Is Vitamin D recommended for Fabry patients?

Richard Corkum

Donna Struss
Vice President

The CFA community is sad to announce the passing of Richard Corkum on September 20th from Bridgewater, N.S. Richard was 52 years old.

Richard actually was the first person to start up the Fabry Association over 20 years ago, and he called it the Fabry Society. He was an advocate for patients in Canada with Fabry disease. He also had a personal involvement with research, development, and testing with the national Institute of health for the disease by donating bone marrow for study purposes.

Richard had a witty sense of humour and always had the ability to make everyone around him laugh.

We are forever grateful for his contribution to Fabry patients. Our thoughts are with his family.

MODIFY - A PHASE 3 STUDY INVESTIGATING A POTENTIAL NEW ORAL TREATMENT FOR FABRY DISEASE

The ongoing MODIFY study is investigating the effectiveness and safety of lucerastat, a potential new oral therapy for Fabry disease. MODIFY is currently enrolling participants at medical centers in North America (USA & Canada), Europe (Austria, Belgium, Germany, Netherlands, Norway, Poland, Spain, Switzerland and United Kingdom) and Australia.

What is neuropathic pain?
a type of pain which feels like burning, shocks or shooting, stabbing, tingling, and/or pins and needles in the hands and feet.
...sound familiar?

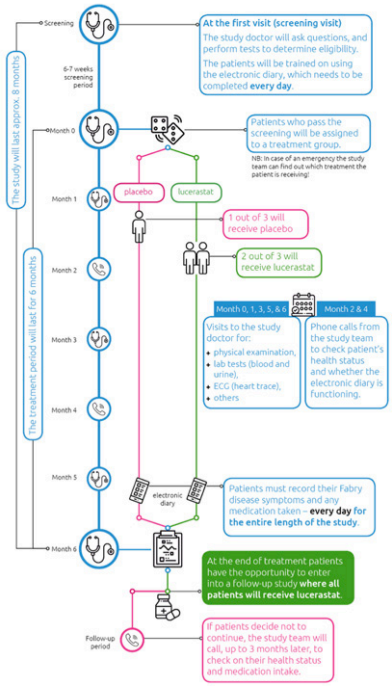
In Canada there are currently 4 actively participating hospitals:
Dr. Ch. Greenberg
in Children's Hospital Research Institute in Winnipeg
Dr. S. Sirrs
in General Hospital in Vancouver
Dr. A. Khan
in University of Calgary
Dr. M. West
in Queen Elisabeth II Health Sciences Centre in Halifax

There are two other sites that just started the study in October:
Dr. D. Bichet
in Hôpital du Sacré-Cœur de Montréal
and site of **Dr. Ch. Prasad**
in London Health Sciences Centre in London, Ontario.

Due to COVID pandemic, the enrollment of new participants may be temporarily affected, based on the situation at each individual hospital.

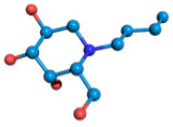
Lucerastat is administered in the form of oral capsules taken twice a day. Any adult with Fabry disease, irrespective of the type of genetic mutation that they have, may be eligible to participate in the MODIFY study. The key entry criterion is neuropathic pain, defined as sensations of burning, shocks or shooting pain, tingling, pins and needles, stabbing, and/or numbness in the hands and feet. Neuropathic pain may be permanent or occur randomly, and may be triggered by heat or cold, a fever, and/or physical activity.

MODIFY is a phase 3 study, meaning that lucerastat is now in the final stage of the clinical trial process that must be completed to evaluate the safety and efficacy of a new medicine before it is submitted to Health Authorities for review.



Participants in the MODIFY study have a 1 in 3 chance of being randomly assigned to receive placebo treatment. The capsules provided to participants assigned to receive placebo treatment will not contain any lucerastat. However, participants who complete the 6-month treatment period will have the option to enroll into an extension study investigating the long-term effects of lucerastat treatment. In the extension study, all participants will receive lucerastat. Treatment in the extension study can be continued for up to 2 years.

The MODIFY study is sponsored by Idorsia Pharmaceuticals Ltd., a pharmaceutical company based in Switzerland (www.idorsia.com). Idorsia will organize and pay for travel, even if study participants live far away from the nearest participating hospital.



For more information about the MODIFY study, visit www.modifyfabry.com (available in both English and French). On the website, patients with Fabry disease can submit a request to be contacted by MODIFY study site teams.

NEW PHASE I/II CLINICAL STUDY TO EXPLORE THE POTENTIAL OF ST-920 INVESTIGATIONAL GENE THERAPY TO TREAT FABRY DISEASE

The STAAR Study is Recruiting Patients Now

Fabry disease is caused by shortage of an enzyme called alpha-galactosidase A (-Gal A). This shortage happens when the GAL gene, which provides the body with instructions for making -Gal A, is not working correctly.

A new phase I/II clinical study has been designed to investigate the safety and tolerability of an investigational gene therapy called ST-920 to treat Fabry disease. ST-920 aims to deliver a healthy copy of the GLA gene to the liver. It is hoped that the liver should then be able to produce the -Gal A enzyme and secrete it via the blood stream to the rest of the body.

The STAAR Study is now recruiting men aged 18 or over who have been diagnosed with Fabry disease. Visit the STAAR Study website (www.staarclinicalstudy.com) where you can see if you qualify. You can also discuss this further with the study team, who are more than happy to help.

Contact details: clinicaltrials@sangamo.com

Toques

Julia Alton
Executive Director

Bundle up this winter with the new Be You Toque!! A special edition item is coming next month - order and wear for Rare Disease Day (February 28 2021). Stay warm and stay tuned.

https://fabrycanada.entripyshirts.com/cfa-knit-toque-be-you-2

PATIENT EMPOWERMENT ANSWER:
A. Yes it is, at least 2000 IU's per day.

Donations to the CFA are greatly appreciated

FALL STRESS REDUCERS

Dr. Seema Kanwal
Board Member

With summer now behind us, fall is by far my most favourite season. The cooler evenings, warm days requiring a sweater, and leaves that have the beautiful red glow brings me pure joy. With this brings for some however, fears around the now cold and flu season upon us. Not to mention the pandemic we are all still in the midst of. Keeping ourselves as healthy as possible is on all of our minds. Let's start with simple things that will help keep our bodies and our minds communicating well, and ultimately, handle stress more effectively.

Let's start by a well-balanced diet that has lots of vegetables (colors of the rainbow and lots of greens), protein, healthy fats and complex carbohydrates. For every meal, aim for vegetables and small amounts of protein. Preparing fruits and pre-cut veggies make great snacks and decrease the tendency to gravitate towards the lesser quality foods. Here are some examples of the food groups:

- **Protein Sources** – plain Greek yogurt, chicken, fish, turkey, lean meats, eggs, beans, lentils, clean protein powder.
- **Vegetables** – green leafy and cruciferous vegetables, anything with lots of color.
- **Healthy Fats** – olive oil, coconut oil, raw nuts and seeds, organic grass fed butter, nut butters.
- **Complex carbohydrates** – grains (1/2 cup), sweet potatoes, yams, jicama and parsnips are a great source of energy for the brain.

When organizing meals, ensure to include high quality sources of healthy fat with each meal such as those listed above. This adds to the satiety value of the meal and reduces the desire to eat a sweet item at the end of the meal. Ideally, you want to aim for 3 main meals and if really needed, then 2 small, balanced snacks will keep blood sugar stable and decrease the desire for sweets and caffeine as well..

If you are someone that does need a snack after dinner, focus on protein options. Ideally, you want to leave 3 hours before bedtime to give your body a chance to rest once you do go to sleep. A good evening snack may include half a cup of plain yogurt, with some chia or hemp hearts or a protein powder mixed into it. This provides protein, some carbs and a healthy source of fat.

Sleep is also another critical component of healthy functioning cellular resiliency. There is a lot of research done over the years of sleeping in a cooler room. Some researchers feel that the perfect temperature for sleeping is around 68 degrees Fahrenheit. A darker room is critical for proper sleep. Now with the sun setting much earlier, this is easier for some, however if you have a light outside your house, shining in, we do need to cover that. Having a dark room is necessary to optimize melatonin output, which is a major anti-oxidant hormone in our body. With what we are all going through at this unprecedented time, stressors of the day can often seem amplified at night while we are lying in the dark ruminating on the day's events. Let them go, nothing will be resolved in the night and will only make tomorrow more difficult for the lack of sleep. Things often seem less stressful in the morning after a good night's sleep.

It is important to take time to play and do the things in life that bring us some type of relief and uplift our spirits. This is one of the best and most rewarding ways of reducing stress. Make a list of the things you like to do that bring a smile into your life. Some things may take ten minutes like walking your dog or some things may take all day like going to your favourite place. It's okay if you are unable to do some of these things during the current time when we are staying home. At least you have put some thought into this and prepare and focus on when we can start doing these activities. Some of these experiences may occur over a weekend or require a week-long vacation. We all must balance work with recovery. Taking the opportunity to get adequate sleep is one of the most important things we can do for ourselves.

Spending time outdoors in nature irrespective of the weather, rain or shine, is a great way to ground us. An afternoon nap or resting quietly is certainly acceptable if possible.

NEW FABRY APP - Participants needed!

M.A.G.I.C. Clinic, and the CFA have collaborated with the makers of ZoeInsights to bring the app to patients as part of a clinical study!

The app will help you stay engaged in your health and stay organized. Track your symptoms, add a caregiver, attach your labs and records so they can all be found in one place!

Available for both iOS and Android, this app allows patients to track symptoms, medications, and much more related to their chronic conditions.

HOW IT WORKS

In this study, we will ask 50% of participants to use the app as they like, creating journal entries whenever they want to, and complete a few questionnaires about their experience. These participants are compared to the other 50%, who are the "placebo" group who do not use the app.

Participants are ELIGIBLE if they are:

- Adult patients (18 years old or older) with a diagnosis of Fabry disease.
- Other inclusion criteria will include **a)** access to a smartphone with data connection; **b)** willingness to devote 10-15 mins of time in a day to log medications and notes, and **c)** able to speak and write English sufficiently to complete questionnaires.

For more information about the app, please visit https://magiccalgary.ca/zoeinsights/ ZoeInsights here.
If you are interested in joining the study, please contact us at researchassistmagic@telus.net or desmond@discoverydna.ca

THANKS TO OUR SUPPORTERS

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

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

Amicus Therapeutics
AVROBIO
Chiesi
idorsia
SANOFI GENZYME
Empowering Life
Takeda

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.