



October 2023 Newsletter

International Gaucher Day 1 October



This year we celebrated International Gaucher Day by releasing a video, [*The Power of Knowledge in a Timely Diagnosis - Building a Better Future*](#), in which patients, caregivers and physicians from different parts of the world discussed their diagnostic journeys.

IGA members and the Gaucher community also shared their own stories on social media

There is a brochure to accompany the video in a variety of languages available on the [IGD page of our website](#).

As well as English subtitles, the video is available with subtitles in the following languages: [Spanish \(Español\)](#), [Serbian \(Srpska\)](#), [Portuguese \(Português\)](#), [French \(Français\)](#), [Russian \(Русский\)](#), [Hindi \(हिंदी\)](#), [Chinese \(中国人\)](#), [Macedonian \(Македонски\)](#) and [Arabic \(عربي\)](#).

EDUCATIONAL WEBINAR

**SUBSTRATE REDUCTION THERAPY:
WHAT WE NEED TO KNOW**

Please join us for 60 minute educational webinar to learn more about oral treatment in Gaucher disease

MONDAY
6 NOVEMBER
REGISTER
[BIT.LY/IGAOTW](https://bit.ly/igaotw)

TIME
6PM CET
LANGUAGES
CROATIAN WITH ENGLISH INTERPRETATION

**Presenter**

Nadira Durakovic, MD PhD
Dept. of Internal Medicine Div. of Hematology
University Hospital Center Zagreb
Zagreb, Croatia

ADVANCE REGISTRATION REQUIRED
For more information and to register visit:
bit.ly/igaotw

ADVANCE REGISTRATION REQUIRED

Gene therapy trials webinars

The Gaucher Community Alliance, in co-operation with the IGA, is presenting a series of webinars on the current open clinical trials for gene therapy in Gaucher disease Type 1 being run by Freeline and Prevail Therapeutics.

The webinars will take place on different days in different regions, and will be interpreted into several languages. The dates are:

- **Europe:** November 21st, 7pm CET (English, Spanish, Italian, French and German)
- **Asia:** November 22nd, 7pm IST (English, Arabic, Hindi, Chinese)
- **Americas:** November 29, 7pm CST (English, Spanish and Portuguese)

For more information and to register, use the buttons below.

Europe

Asia

Americas

LEAP2MONO webinars online



Raising awareness of the GD3 Venglustat trial

**The LEAP2MONO trial for type 3 Gaucher disease:
building on scientific foundations, phase 2 trial results and study design.**

Speaker - Dr. Pramod Mistry

Please use the Q&A box to ask questions that will be answered at the end of the webinar.

This webinar will be recorded and uploaded to our Youtube, please make sure not to include any personal information in your questions for this reason.

The IGA recently held a series of webinars to raise awareness of the Gaucher disease Type 3 Venglustat trial titled: ***The LEAP2MONO trial for type 3 Gaucher disease: building on scientific foundations, phase 2 trial results and study design.***

The webinars were presented by Dr. Pramod Mistry MBBS, PhD, MA, MD, Professor of Medicine (Digestive Diseases) and of Pediatrics (Gastroenterology); Professor of Cellular & Molecular Physiology; Director of Yale Lysosomal Disease Center and Gaucher Disease Treatment Center, and one of the Principal Investigators for the study.

The videos of the three webinars are now online. View them using the buttons below. We will be adding subtitles in different languages in the coming weeks.

Europe

Asia

Americas



Report from the 5th regional Gaucher meeting in Zagreb

From September 29th till October 1st, 2023, the 5th regional Gaucher meeting was held in Zagreb, organized by the Croatian Gaucher Association. It is already a traditional gathering, the fifth in a row, after Čatež (2012), Sarajevo (2014), Belgrade (2016) and Struga (2019). The meeting was attended by patients, their family members, doctors and nurses from Croatia, Slovenia, Serbia, Bosnia and Herzegovina, Montenegro and North Macedonia.

IGA Chair Biljana Jovanović, who was present at the gathering, writes: "The 5th Regional Gaucher Patients Meeting in Zagreb was a successful event that enhanced understanding, solidarity, and cooperation among patients and their communities in the region. The Meeting also provided an opportunity for the exchange of real-life stories and experiences among patients and their families. This exchange of experiences and support was invaluable for each participant, helping us feel less isolated and better informed about the condition.

"I must note that Jasenka Wagner did an exceptional job in organizing the event. Her dedication and attention to detail ensured the smooth and successful execution of the meeting. It is my honour and pleasure to have been a part of this remarkable event."

[Read Jasenka's full report](#)

Gaucher Awareness Day in Nepal



A Gaucher awareness day in Kathmandu, Nepal as part of their International Gaucher Day (IGD) celebrations. Held on the 12th of October and organised by IGA regional manager Nirmal Khadka, with the help of the Rare Disease Society Nepal and the Muscular Dystrophy Association. the focus on raising awareness of Gaucher diseases. More than

80 people attended the event among whom there were paediatric neurologists, geneticists, endocrinologists, government, media, World Health Organization and patient representatives, parents, and patients. Also, the former mayor and a famous artist were present.

At the beginning of the event the IGD video was played and then three speakers gave short lectures on Gaucher disease (treatment, symptoms, diagnosis, need for cooperation, and improving quality of life). Discussion followed to come to conclusions and recommendations.

Nirmal said, "We do believe that through continuous education and raising awareness we can contribute to improving the lives of families with Gaucher disease in Nepal and our neighbouring countries in the region."

The Himalayan Times published a report.

[Read it here.](#)



The founders of the Gaucher Community Alliance, an IGA member in the USA, recently took part in a television show about Gaucher disease. Among other topics, they spoke about the importance of patient advocacy in improving the lives of patients and their caregivers.

Volunteer Spotlight

Roselyn Odero

Roselyn Odera (Kenya) decided to volunteer for the position of IGA Africa Regional Manager because she felt that Gaucher is under reported in Africa and being one of the few rare diseases with some treatment options, awareness would help both clinicians and patients' families obtain an early diagnosis which would hopefully lead to early interventions. She was also interested in working with an international rare disease organization to learn new skills in the field.



Please contact Vesna if you are interested in volunteering with us.

International Gaucher Alliance

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