

29 November 2023 - 30 November 2023

# 10<sup>TH</sup> EUROPEAN TAY-SACHS & SANDHOFF FAMILY CONFERENCE



SUPPORTED BY

[azafaros](#)

[sanofi](#)

# CONTENTS

The full agenda of the 10th European Tay-Sachs and Sandhoff Family Conference is below, and you can watch each of the presentations given by our speakers over the two days on YouTube via the summary page for each talk

|   |   |
|---|---|
| European Tay-sachs & Sandhoff charity consortium (ETSCC)..... | 3 |
| The conference.....   | 4 |

## DAY 1

|   |    |
|---|----|
| Day 1 introduction.....   | 7  |
| Presentation 1 – Introducing the ETSCC and a review of the latest projects launched by its members .....        | 8  |
| Presentation 2 – The first year after diagnosis: The clinician perspective .....                                | 9  |
| Presentation 3 – The first year after diagnosis: The industry perspective .....                                 | 10 |
| Presentation 4 – The first year after diagnosis: The family perspective .....                                   | 11 |
| Presentation 5 – Introducing the Clinical Toolkit for Tay-Sachs and Sandhoff .....                              | 12 |
| Panel – How can we make sure that families get the right support in the first 12 months after a diagnosis? .... | 13 |
| Presentation 6 – Experience of caregivers of individuals living with GM2 / GM1 gangliosidoses.....              | 14 |
| Presentation 7 – How advocacy can manage the cultural differences in Europe .....                               | 15 |
| Presentation 8 – A familiar story in the first 12 months.....   | 16 |
| Panel – Gap analysis: what are we doing well and what else do we need to be doing? .....                        | 17 |
| Day 1 summary .....   | 18 |

## DAY 2

|   |    |
|---|----|
| Day 2 introduction.....   | 20 |
| Presentation 9 – Review on the research into Tay-Sachs and Sandhoff .....                                 | 21 |
| Presentation 10 – Updates on the development of IB1001 for GM2 gangliosidoses .....                       | 22 |
| Presentation 11 – Overview of the work for GM2 by JCR Pharmaceuticals .....                               | 23 |
| Presentation 12 – 8 in 1stroke.....   | 24 |
| Presentation 13 – Clinical update: The journey of Nibaglustat .....                                       | 25 |
| Presentation 14 – Bringing patients insights to understand meaningful clinical relevance .....            | 26 |
| Presentation 15 – From trial design to clinical relevance.....  | 27 |
| Presentation 16 – Construction and validation of the infantile onset GM2 gangliosidosis rating scale..... | 28 |
| Presentation 17 – Introducing the C-POS .....   | 29 |
| Presentation 18 – Introducing the Global GM1 and GM2 Alliance (GGGA).....                                 | 30 |
| Day 2 summary .....   | 31 |

|                           |    |
|---------------------------|----|
| ETSCC goals for 2024..... | 32 |
|---------------------------|----|

# EUROPEAN TAY-SACHS & SANDHOFF CHARITY CONSORTIUM (ETSCC)

The ETSCC was established in 2013 to enable European charities to come together in the fight against Tay-Sachs and Sandhoff. The member charities are from the United Kingdom, Spain, France, Germany and Portugal.

All of the patient advocacy groups who are members of the consortium are recognised charities in their country of origin. The three aims of the ETSCC are to raise awareness of Tay-Sachs and Sandhoff, support the research for a potential treatment and provide a united European voice. The members of the ETSCC meet once a year to discuss the progress of the research into Tay-Sachs and Sandhoff and the consortiums strategy.

The members of the ETSCC are The Cure & Action for Tay-Sachs (CATS) Foundation based in the UK; Acción y Cura para Tay-Sachs (ACTAYS) based in Spain; Vaincre Les Maladies Lysosomales (VML) based in France; Hand in Hand gegen Tay-Sachs und Sandhoff based in Germany; and Divulgar e Orientar para Combater e Enfrentar a Tay Sachs, Sandhoff e GM1 (DOCE) based in Portugal.

The ETSCC was formed to give a European voice to patients, families and charities battling to find a cure for Tay-Sachs and Sandhoff disease. We do this by:

**RAISING AWARENESS** - Members of the ETSCC are all dedicated to raising awareness and creating a deeper understanding of Tay-Sachs and Sandhoff disease.

**SUPPORTING RESEARCH** - The ETSCC supports the research into Tay-Sachs and Sandhoff and helps drive forward these projects so a cure can be found.

**UNIFIED VOICE** - As a collective, the ETSCC allows for country specific charities supporting Tay-Sachs and Sandhoff have a unified voice against the diseases.



## ETSCC ADVOCACY PARTNERS



# THE CONFERENCE

The 10th European Tay-Sachs and Sandhoff Family Conference is the third event we have held online to bring together all our families and partners to hear about the latest work into supporting our community

In Europe we have worked hard at bringing Tay-Sachs and Sandhoff families together so that they have access to information and support. Through the ETSCC we have been holding annual family conferences with the intention to provide a forum for discussion about topics that can support the care of someone affected by the diseases, to hear updates on the latest research into treatments and enable families to meet each other in person.

The first event was held in Mondsee in Austria in 2013 and was attended by four families. This event was the start of something which has grown year on year and we have held events in London, Madrid, Paris and Wurzburg. However, due to the COVID crisis that has impacted the entire world we were forced to shift how these events have been held. Instead of an in-person conference where families have been able to meet, we had to move online for the last two years.

After the success of the last two conferences held virtually, we were delighted that in 2023 we were able to hold our 10th European Tay-Sachs and Sandhoff Family Conference. Utilising the digital format, we have been able to reach more families who could

not travel to an in-person event. In addition to this, we have been able to secure speakers from various companies who may have not been able to attend a live conference.

We are aware that the in-person conference is preferable to our families. We also know that a digital version makes it accessible to more people, so we are planning on changing the format on how our Family Conferences are held in the future. We plan to explore holding an in-person event every two years in the Spring / Summertime which will have a focus on families meeting and learning about caring for someone impacted by Tay-Sachs and Sandhoff. In addition to this, we plan to hold an annual virtual conference in November / December of each year to hear summary talks from industry and new projects being launched by advocacy groups.

We hope that this new format will enable us to continue our reach of support to families whilst also giving our industry and advocacy partners the opportunity to continue working with our very special community all year around so we can continue to provide support.

---

## EMMA MURPHY - MUM TO STANLEY

"Every year we continue to learn new things about our community, not only about the research and trials that are being conducted but also in the ways in which support is always a priority for the different advocacy groups."

## TAYLOR FIELDS - INTRABIO

"We always attend the ETSCC event as we believe it is vital that we continue to show support to the community, not only with the information we provide in a transparent way, but also to show them we are always present providing support where we can."

The agenda for the 10th European Tay-Sachs and Sandhoff Family Conference was developed based on ensuring that we covered topics that were of value to our community. We wanted to ensure that there was the opportunity for our families to learn about projects that have been launched for Tay-Sachs and Sandhoff disease whilst hearing the latest research updates. We achieved this by continuing to split the conference over two days, where each day has a specific focus.

Day one covered the topic of understanding the challenges faced by families in the first 12 months after diagnosis and how we ensure that they have access to the best support, regardless of where they live. This day also highlighted some of the projects that the ETSCC member organisations had completed to support the Tay-Sachs and Sandhoff. The second day of the conference focussed on the research and current studies for Tay-Sachs and Sandhoff along with the work that is being done to develop a truly global movement for our community which has the potential to reach communities who do not have any national support in place.



[VISIT THE CONFERENCE WEBSITE](#)

**THE 10TH EUROPEAN TAY-SACHS AND SANDHOFF FAMILY CONFERENCE WAS HELD USING THE HOPIN TECHNOLOGY WHICH ALLOWED US TO REACH AN AUDIENCE ALL OVER THE WORLD**

Every year we are very grateful to all the speakers and companies who agree to attend and present at the conference. They all generously give up their time to provide updates on their work and detail how this is impacting the Tay-Sachs and Sandhoff community. We also want to say a big thank you to Azafaros and Sanofi who sponsored the 10th European Tay-Sachs and Sandhoff Family Conference. Their support was vital in enabling us to hold the event in 2023.



# 52%

## PARENT, CARER AND PATIENT PARTICIPANTS

Attendees from this group were from 15 different countries.

# 35%

## INDUSTRY PARTICIPANTS 3

Seven different industry companies were in attendance.

# 9%

## ADVOCACY PARTICIPANTS 9

Attendees from ten different advocacy groups joined the conference.

# 9%

## CLINICIAN PARTICIPANTS 9

Clinicians from five countries joined the event.

# 3%

## RESEARCHER PARTICIPANTS 3

Researchers based in the US and Europe attended the conference.



# 18 PRESENTATIONS 2 PANEL DISCUSSIONS

### REGIONAL SPLIT OF CONFERENCE PARTICIPANTS

|               |     |
|---------------|-----|
| Europe        | 64% |
| North America | 29% |
| Asia          | 2%  |
| South America | 2%  |
| Australasia   | 1%  |
| Middle East   | 1%  |



#### Follow this Tip:

Do you want to read the complete report? This will be available on the ETSCC website alongside summaries of other conferences.

[CLICK HERE TO READ THE EVENT SUMMARY FROM ALL OUR CONFERENCES](#)



# DAY 1

# INTRODUCTION

The first 12 months after a diagnosis play a big part in the ways in which families can come to terms with a diagnosis. Access to information and understanding the course of the disease can help families not only plan for the future, but also help them put in place various support mechanisms as and when they are required.

During the first session of day one at the conference there were various speakers from different backgrounds discussing the challenges and potential solutions for the first 12 months after someone is diagnosed with Tay-Sachs or Sandhoff disease. Speakers including clinicians, industry, advocacy and families shared their views and experiences on the topic. The panel then delved into discussing what families may be lacking in those first 12 months after diagnosis and how additional support can be provided.

The second part of the first day of the conference provided speakers who discussed the role and challenges of caregivers. The speakers, from a mix of advocacy and industry, dived into the topic and identified the ways in which caregivers play a vital role in providing support to those people impacted by Tay-Sachs or Sandhoff disease. The final panel was focussed on a gap analysis of what is missing in terms of support and how the key stakeholders in this topic can work together to fill this void. You can watch the presentations on YouTube via the button on each summary page.

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### Highlighting the importance of the first 12 months after diagnosis

“Hearing about the historical challenges that other families had in the first 12 months after diagnosis and how many of the advocacy groups have addressed this has really shown me how far the community has come.”

*Conference attendee*

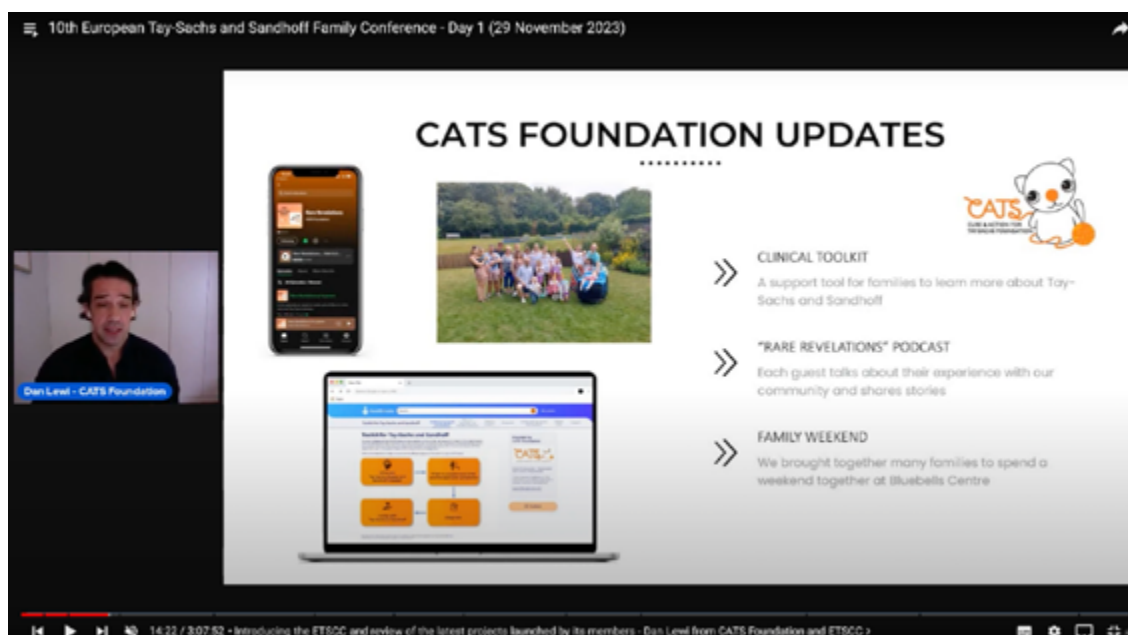


# PRESENTATION 1

## 13:05 - 13:15

### Introducing the ETSCC and a review of the latest projects launched by its members

Dan Lewi provided an overview of the ETSCC, its history and what its goals are. The talk also highlighted the projects that each of the ETSCC members have completed in the last 12 months and the impact these have had on the Tay-Sachs and Sandhoff community.



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### DAN LEWI

Chairman of the ETSCC & Co-founder of the Cure & Action for Tay-Sachs (CATS) Foundation

To watch the [presentation click here](#)

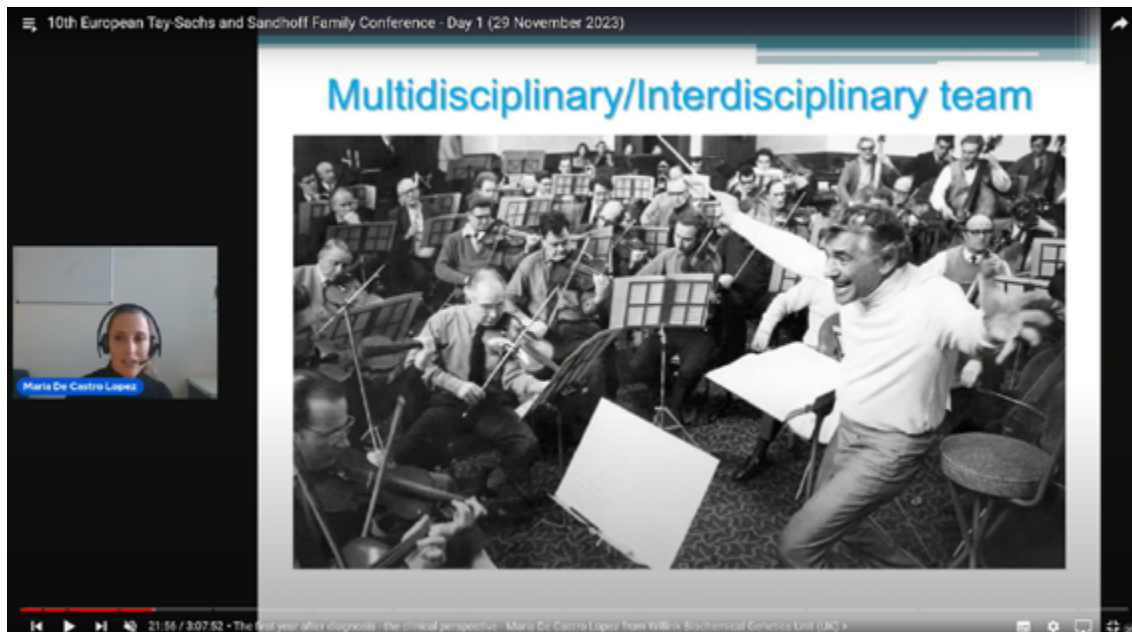


# PRESENTATION 2

## 13:15 - 13:30

### The first year after diagnosis: The clinician perspective

Dr Maria De Castro Lopez gave a talk about the role clinicians play in helping and supporting families as they plan to navigate the first 12 months after diagnosis to ensure they get the support services they need in place.



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### DR MARIA DE CASTRO LOPEZ

Consultant in Paediatric Inherited Metabolic Diseases at Willink Biochemical Genetics Unit

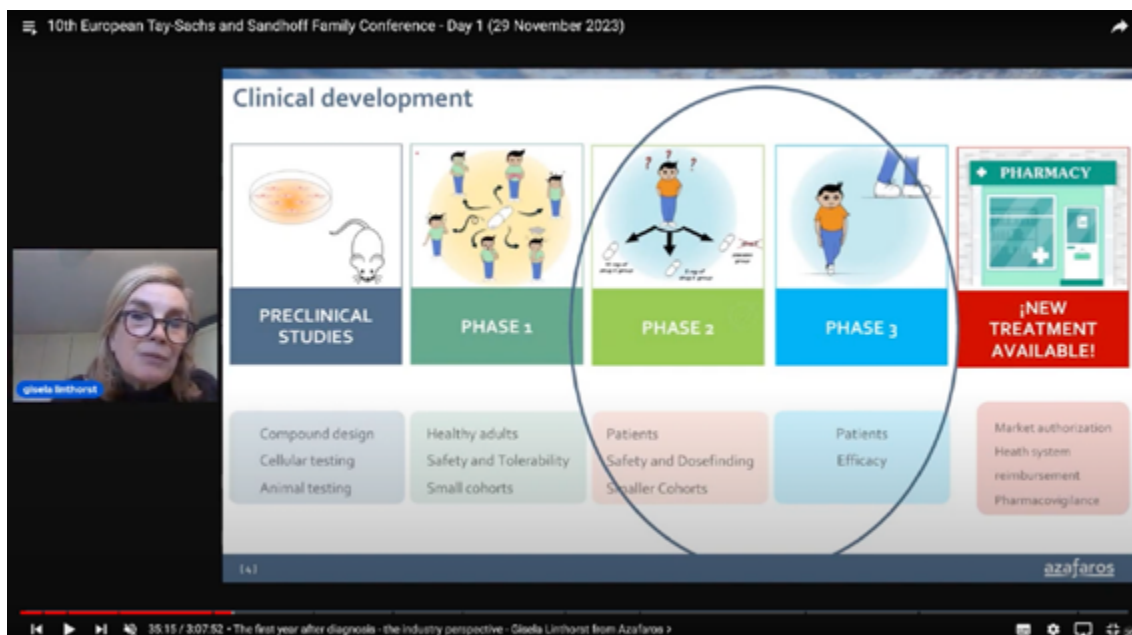
To watch the [presentation click here](#)

# PRESENTATION 3


## 13:30 - 13:45

### The first year after diagnosis: The industry perspective

Gisela Linthorst talked through the ways in which industry can provide information and signposting to families after a diagnosis of Tay-Sachs and Sandhoff to ensure they are provided up to date information on the diseases's development of treatments.



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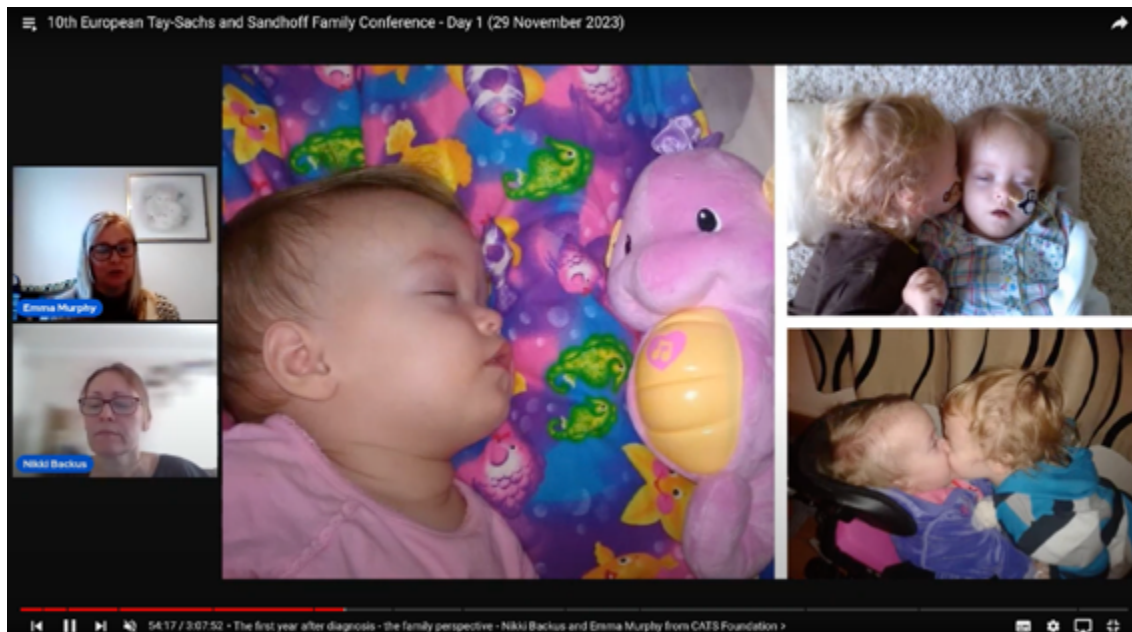
**GISELA LINTHORST**  
 Head of Patient advocacy and Patient Engagement at Azafaros  
 To watch the [presentation click here](#)

# PRESENTATION 4

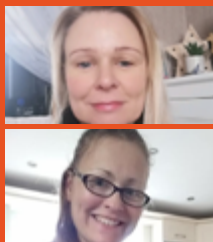
## 13:45 - 14:00

### The first year after diagnosis: The family perspective

Nikki and Emma talked through their families experiences of the first 12 months after a diagnosis of Tay-Sachs and Sandhoff and highlighted what helped them during this period to fully understand the impact of the diseases.



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### EMMA MURPHY & NIKKI BACKUS

Family Support Officers at The CATS Foundation

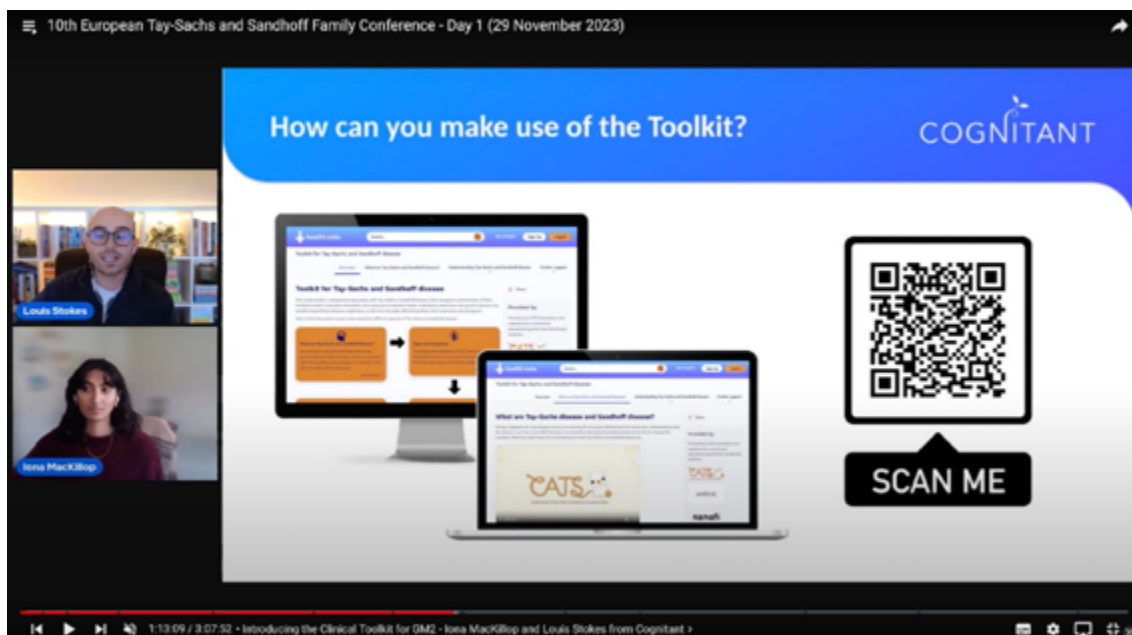
To watch the [presentation click here](#)

# PRESENTATION 5

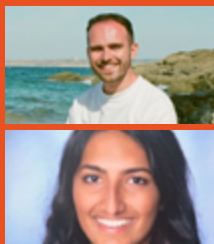
## 14:00 - 14:15

### Introducing the Clinical Toolkit for Tay-Sachs and Sandhoff disease developed by The CATS Foundation

Louis and Iona from Cognitant presented the new Clinical Toolkit for Tay-Sachs and Sandhoff disease that was developed alongside The CATS Foundation where a co-creation methodology with families, clinicians and advocacy was used to ensure the content met the needs of those people using it.



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#### LOUIS STOKES & IONA MACKILLOP

Senior Researcher & Medical Writer at Cognitant

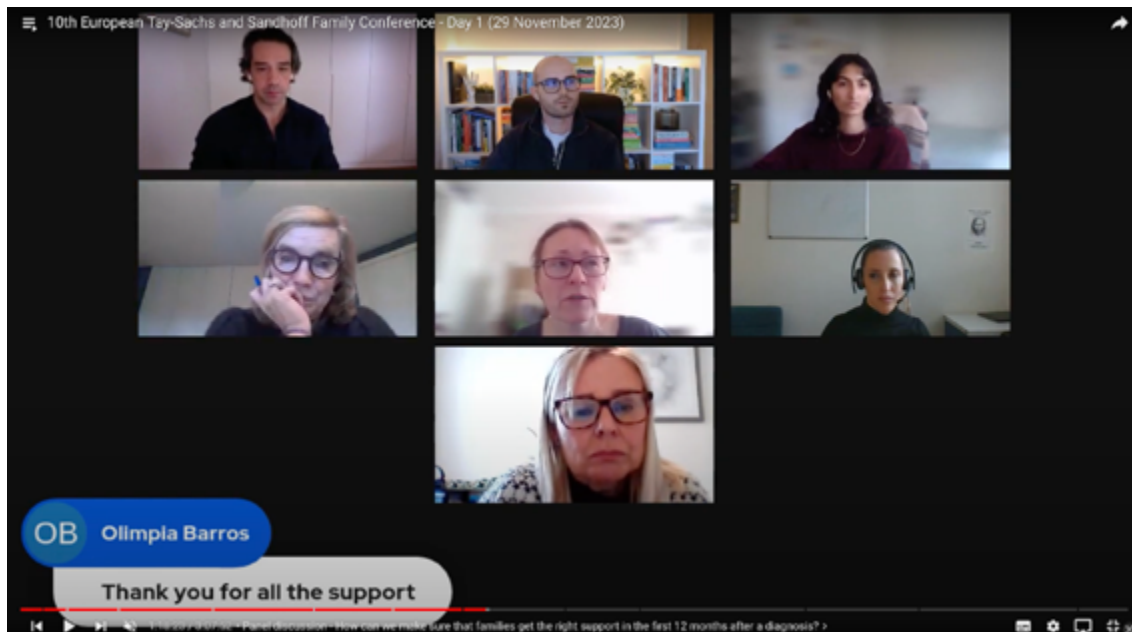
To watch the [presentation click here](#)

# PANEL DISCUSSION

## 14:15 - 14:30

### How can we make sure that families get the right support in the first 12 months after a diagnosis?

The speakers from the first section of the day discussed the tools that families need in the first 12 months after diagnosis and why it is vital to ensure that they are provided with as much support in this critical moment in their life.



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### PANEL ATTENDEES

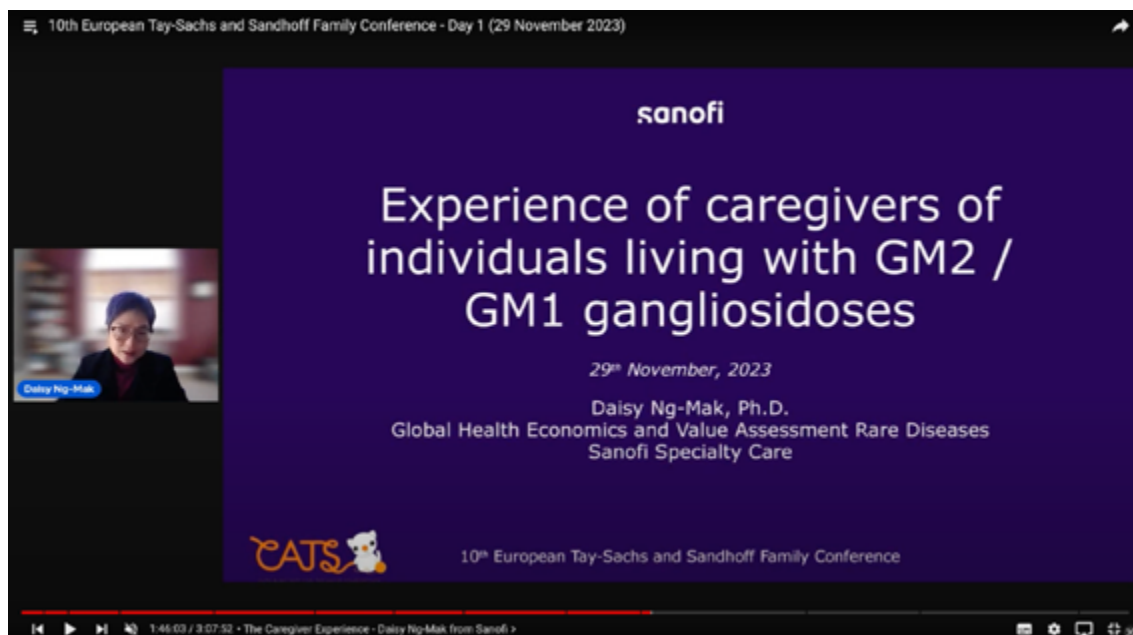
Dan Lewi; Maria De Castro Lopez; Gisela Linthorst; Nikki Backus; Emma Murphy; Louis Stokes; and Iona MacKillop

# PRESENTATION 6

## 14:40 - 15:10

### Experience of caregivers of individuals living with GM2 / GM1 gangliosidoses

Dr. Daisy Ng-Mak gave a talk that detailed the work that Sanofi has completed at looking into the caregiver experience to highlight how this is an important aspect that needs to be considered both in terms of time post diagnosis and in developing treatments.



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**DAISY NG-MAK**

GM2 HEVA Business partner at Sanofi

To watch the [presentation click here](#)

# PRESENTATION 7

## 15:10 - 15:25

### How advocacy can manage the cultural differences in Europe

Maria will talk through the work that EURORDIS have done in supporting families and how they manage the cultural differences throughout Europe in relation to that support with the goal to empower advocacy groups to ensure they have the tools they need.



10th European Tay-Sachs and Sandhoff Family Conference - Day 1 (29 November 2023)

#### International action was there from the beginning

EURORDIS-Rare Diseases Europe was created in 1997 by four founding members

- the Association Française contre les Myopathies, AIDES Fédération Nationale, Vaincre La Mucoviscidose and La Ligue Nationale Contre le Cancer.
- Our original goal was to advocate for EU laws to bring medicines to people living with a rare disease.



EURORDIS  
Rare Diseases Europe

EURORDIS.ORG

EURORDIS  
RARE DISEASES EUROPE

2:15:55 / 3:07:52 • Managing the cultural differences in Europe - Maria Cavaller from EURORDIS

[CLICK HERE TO WATCH](#)



#### MARIA CAVALLER

Patient Engagement at EURORDIS

To watch the [presentation click here](#)



# PRESENTATION 8

## 15:25 - 15:40

### A familiar story in the first 12 months

Allan from the Pompe Support Network will talk through the challenges the Pompe community face in the first 12 months and reflect on the similar journey they face that many of our Tay-Sachs and Sandhoff community have encountered.

10th European Tay-Sachs and Sandhoff Family Conference - Day 1 (29 November 2023)

### Personal experience with Pompe

- Son born 1989, diagnosed 1991.
- Juvenile Onset
  - Floppy baby (premature)
  - Frequent chest infections
  - Airway obstructions
- Respiratory arrest age 2 led to diagnosis at GOSH
- Books said life expectancy <20 years
- Scoliosis correction at 14 (2003)
- Clinical trial in Rotterdam for ERT at 15 (2004-6)
- Stable since and now a successful short-film director
  - Directed the 5 short films on our website.
  - Father of healthy 20-month-old son
- Life is good!

29/11/2023

**POMPE**  
SUPPORT NETWORK

www.pompe.uk

2:36:12 / 3:07:52 • A familiar story in the first 12 months - Allan Muir from Pompe Support Network UK

[CLICK HERE TO WATCH](#)



### ALLAN MUIR

Trustee and Founder at Pompe Support Network

To watch the [presentation click here](#)

# PANEL DISCUSSION

## 15:40 - 15:55

### Gap analysis: what are we doing well and what else do we need to be doing?

The final panel of the day from the speakers who presented during the second part of the day talked through what we are doing well as a community in the first 12 months after diagnosis and what we need to do better to make sure people have the support they need.



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**PANEL ATTENDEES**

Dan Lewi; Emma Murphy; Allan Muir; Maria Cavaller; and Daisy Ng-Mak

# SUMMARY

## DAY 1

A day that focused on the first 12 months after diagnosis in the GM2 community.

The presentations and panels from the first day really highlighted all the work that has been done over the last decade at ensuring families have access to support and information during the first 12 months after diagnosis. Learning about the new tools that have been created by advocacy to enhance support is another indication that there is an ever changing need to keep support services up to date. The Tay-Sachs and Sandhoff community deserves to have the highest level of support, and the first day of the conference detailed how they are now receiving this.



[CLICK HERE TO WATCH THE SUMMARY](#)



SUPPORT CONTINUES TO BE  
REVISED

**29%**

UK based attendees



FUNDING WILL ALLOW  
SUPPORT TO CONTINUE

**9%**

French based attendees



SUPPORT IS ALWAYS  
INNOVATIVE

**9%**

Portugal based attendees

# DAY 2

# INTRODUCTION

Keeping our community up to date on the latest research being conducting in Tay-Sachs and Sandhoff is vital as it enables all our families to see the progress that is being made as we work towards treatment options.

As with last year, there were many ups and downs in the research for treatments for Tay-Sachs and Sandhoff disease. More companies sadly confirmed their exchanges from the space, and this left the community deflated. However, there are still treatments being developed and these studies have been able to continue at pace and are providing real hope to the community. There is a belief that not only will these provide hope to families, they will provide breakthroughs for other research opportunities.

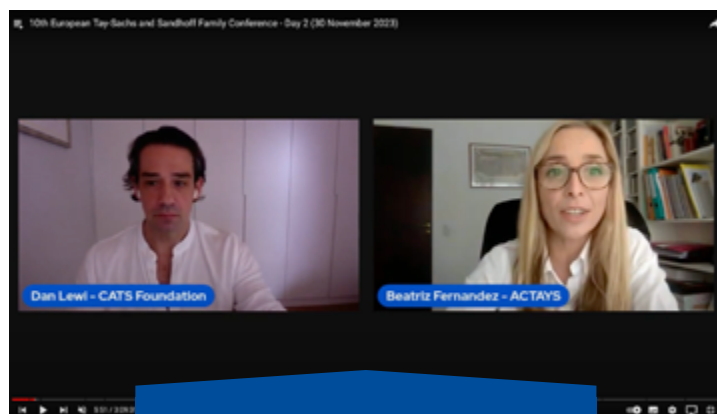
For the second day of the conference, the companies who are developing treatments and research ideas joined the event to provide updates on their work and to share information on topics related to clinical trials. The session was used as an educational opportunity so that those participating could understand in greater detail the research that is being undertaken and how this is being done. You can watch the presentations on YouTube via the button on each summary page.

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## Research into Tay-Sachs and Sandhoff continues to progress

*"Watching the progress of the research has been tough. It's been really difficult to see companies withdrawn from our community but I remain hopeful that one day we will have a treatment for Tay-Sachs and Sandhoff."*

*Conference attendee*



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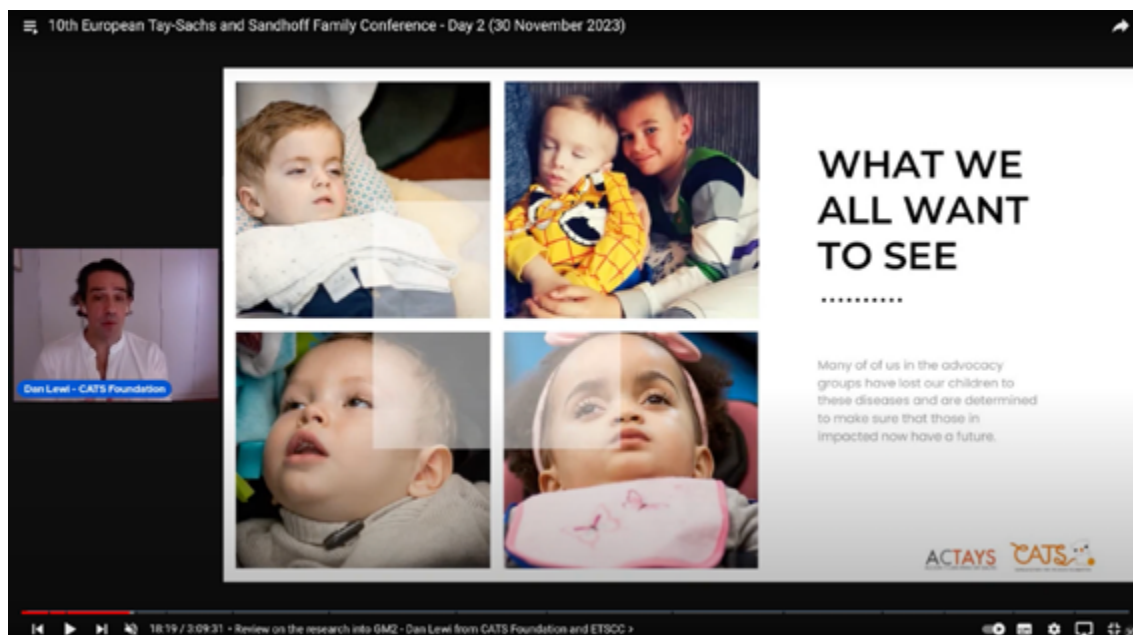


# PRESENTATION 9

## 13:10 - 13:20

### Review on the research into Tay-Sachs and Sandhoff

Dan provided a review of why research into potential treatments is so important for the Tay-Sachs and Sandhoff community and how we work that each of the advocacy groups does industry is vital as we support their work.



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### DAN LEWI

Chairman of the ETSCC & Co-founder of the Cure & Action for Tay-Sachs (CATS) Foundation

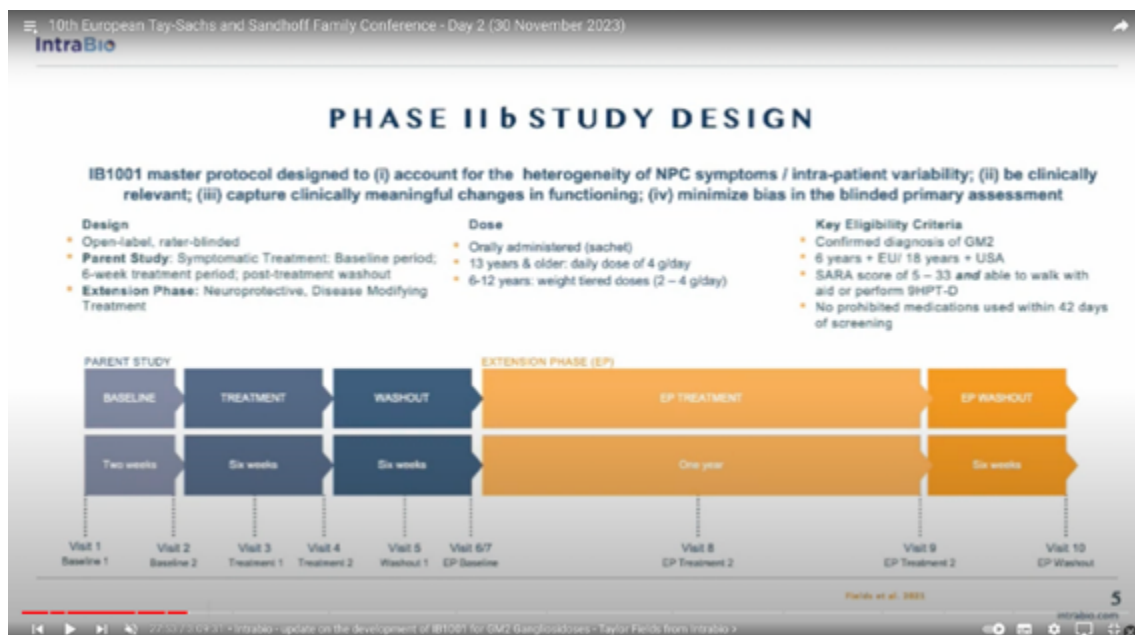
To watch the [presentation click here](#)

# PRESENTATION 10

## 13:20 - 13:40

### Updates on the development of IB1001 for GM2 gangliosidoses

Taylor Fields provided an update on the IB1001 trial for Tay-Sachs and Sandhoff. The talk focused on the plans for the study in the next 12 months and how their work in Niemann-Pick C has also been progressing in the clinic.



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**TAYLOR FIELDS**

Chief Product Development Officer at IntraBio

To watch the [presentation click here](#)

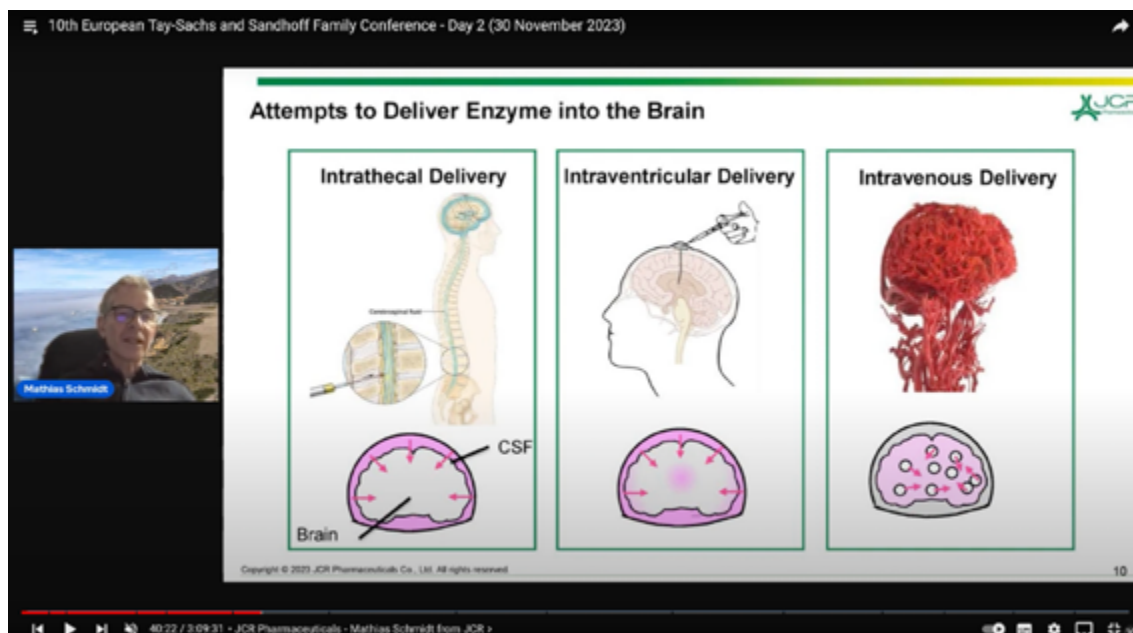


# PRESENTATION 11

## 13:20 - 13:40

### Overview of the work for GM2 by JCR Pharmaceuticals

Mathias Schmidt gave a presentation which provided an update from JCR Pharmaceuticals to the Tay-Sachs and Sandhoff community in Europe. The talk gave an overview of the work they are undertaking to develop a potential treatment for our community.



10th European Tay-Sachs and Sandhoff Family Conference - Day 2 (30 November 2023)

#### Attempts to Deliver Enzyme into the Brain

**Intrathecal Delivery**  
Diagram showing a needle inserted into the spinal canal to deliver enzyme into the cerebrospinal fluid (CSF). Labels include 'Cerebrospinal fluid' and 'Brain'.

**Intraventricular Delivery**  
Diagram showing a needle inserted into the brain ventricle to deliver enzyme. Labels include 'Brain' and 'CSF'.

**Intravenous Delivery**  
Diagram showing a needle inserted into a blood vessel to deliver enzyme. Labels include 'Brain' and 'CSF'.

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#### MATHIAS SCHMIDT

President & CEO of JCR USA, Inc.

To watch the [presentation click here](#)

# PRESENTATION 12

## 13:40 - 14:25

### 8 in 1 stroke

Dr Mengel provided an update on their 8 in 1 study being run at the SphinCS clinic which is focussing on developing a deeper understanding of Tay-Sachs and Sandhoff disease and to support the identification of new endpoints for the diseases.



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### EUGEN MENGEL

CEO & PI at SphinCS

To watch the [presentation click here](#)

# PRESENTATION 13

## 14:25 - 14:40

### Clinical update: The journey of Nibaglustat

Laura from Azafaros provided a clinical update which detailed the journey of Nibaglustat, including an update on the PRONTO study and how advocacy has been supporting their work as they move forward with the trials.



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**LAURA LOPEZ DE FRUTOS**

Medical Affairs at Azafaros

To watch the [presentation click here](#)

# PRESENTATION 14

## 14:40 - 14:55

### Innovative trial design: Bringing patients insights to understand meaningful clinical relevance

Isabela from Sanofi gave an interesting presentation that focussed on the insights that patients can give to support the development of clinical trials that impact both them and the wider Tay-Sachs and Sandhoff community in the future.



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### ISABELA BATSU

Global Project Head, Clinical Development, Rare Diseases at Sanofi

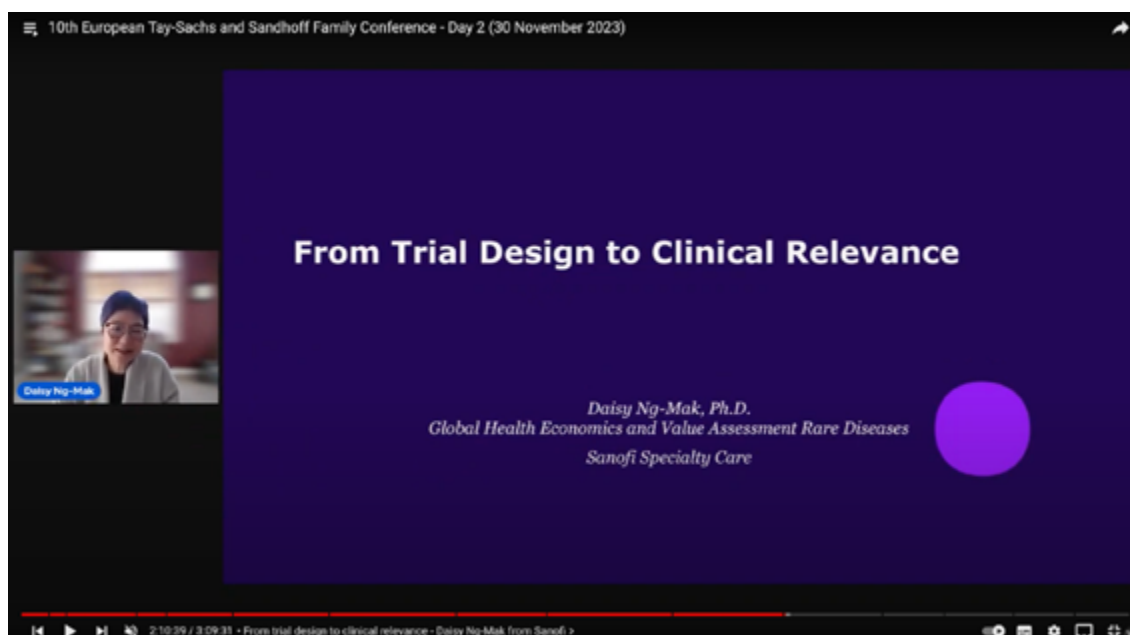
To watch the [presentation click here](#)

# PRESENTATION 15

## 14:55 - 15:10

### From trial design to clinical relevance

Daisy from Sanofi explored the topic of how trial design informs clinical relevance. The presentation touched on the role of many different stakeholders who are involved in the trial design and the impact they have on patient communities.



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**DAISY NG-MAK**

GM2 HEVA Business partner at Sanofi

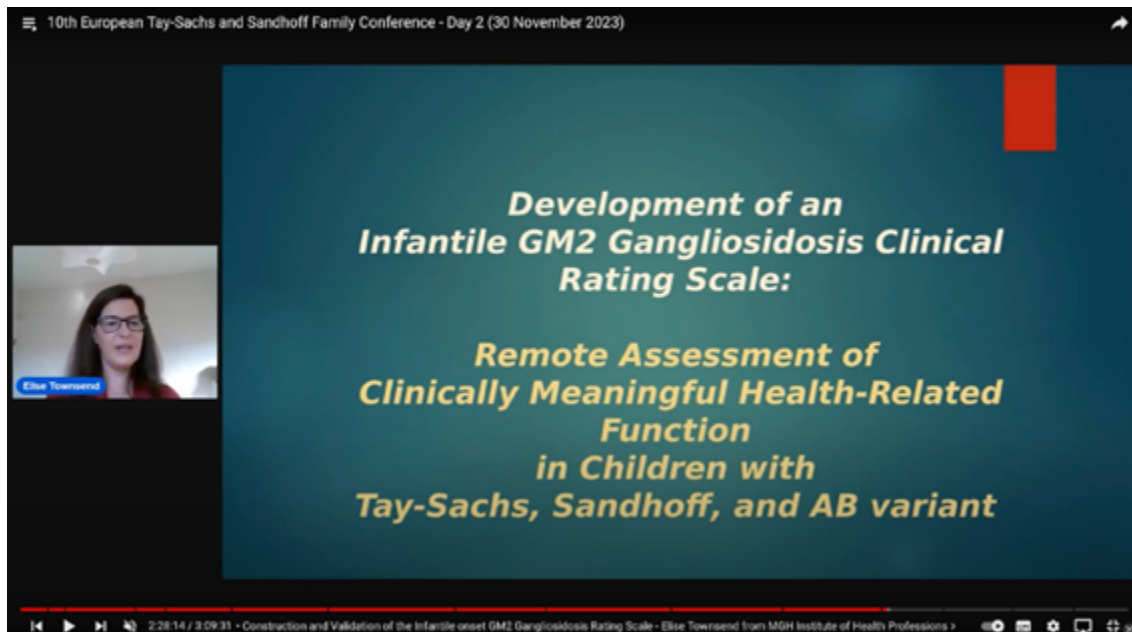
To watch the [presentation click here](#)

# PRESENTATION 16

## 15:10 - 15:25

### Construction and validation of the infantile onset GM2 gangliosidosis rating scale

Dr Townsend gave an update on the study being conducted to develop a virtual clinical assessment tool for Infantile onset GM2. The talk focused on the scale and how a disease specific tool can be used to assess potential treatments.



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#### ELISE TOWNSEND

Associate Professor and Admissions Chair, Department of Physical Therapy at MGH Institute of Health Professions

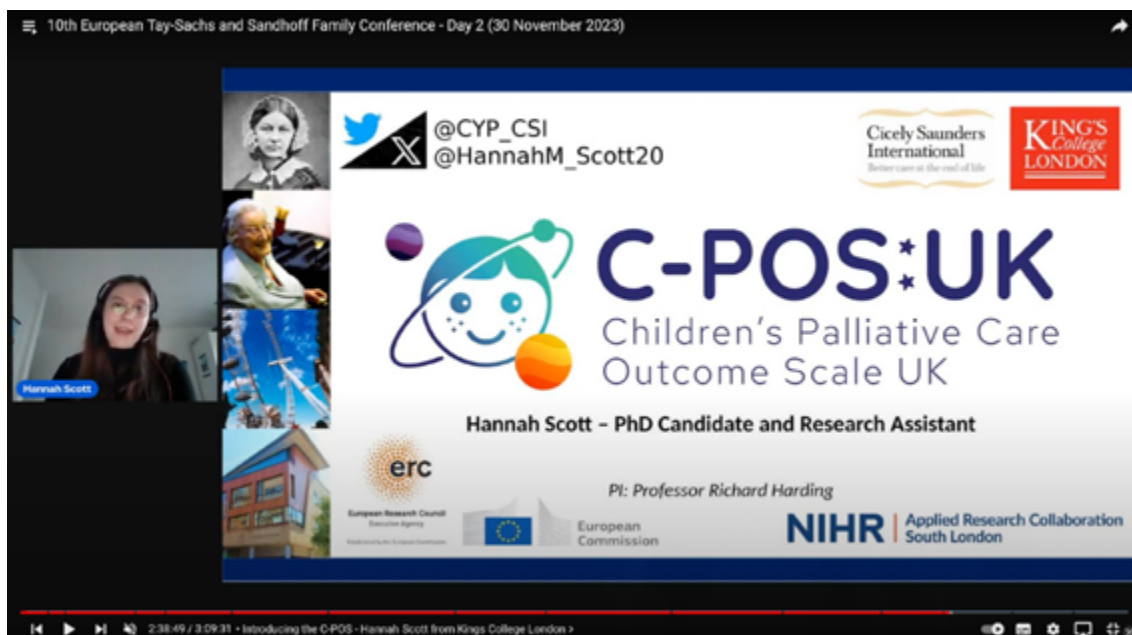
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# PRESENTATION 17

## 15:25 - 15:40

### Introducing the C-POS

Hannah introduced the Children's Palliative Care Outcome Scale (C-POS), a new tool that is being developed at Kings College London. The ways in which the scale is being constructed was discussed and how it could be used in the future.



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### HANNAH SCOTT

PhD Candidate and Research Assistant at Kings College London

To watch the [presentation click here](#)



# PRESENTATION 18



## 15:40 - 15:50

### Introducing the Global GM1 and GM2 Alliance (GGGA)

Kathy and Dan introduced the new initiative that is being launched by all the advocacy groups for GM1 and GM2. The recently formed GGGA will serve as an umbrella organisation that will support regions with little or no support available to them.



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**KATHY FLYNN & DAN LEWI**

CEO at the NTSAD; and Chairman of the ETSCC & Co-founder of the Cure & Action for Tay-Sachs (CATS) Foundation

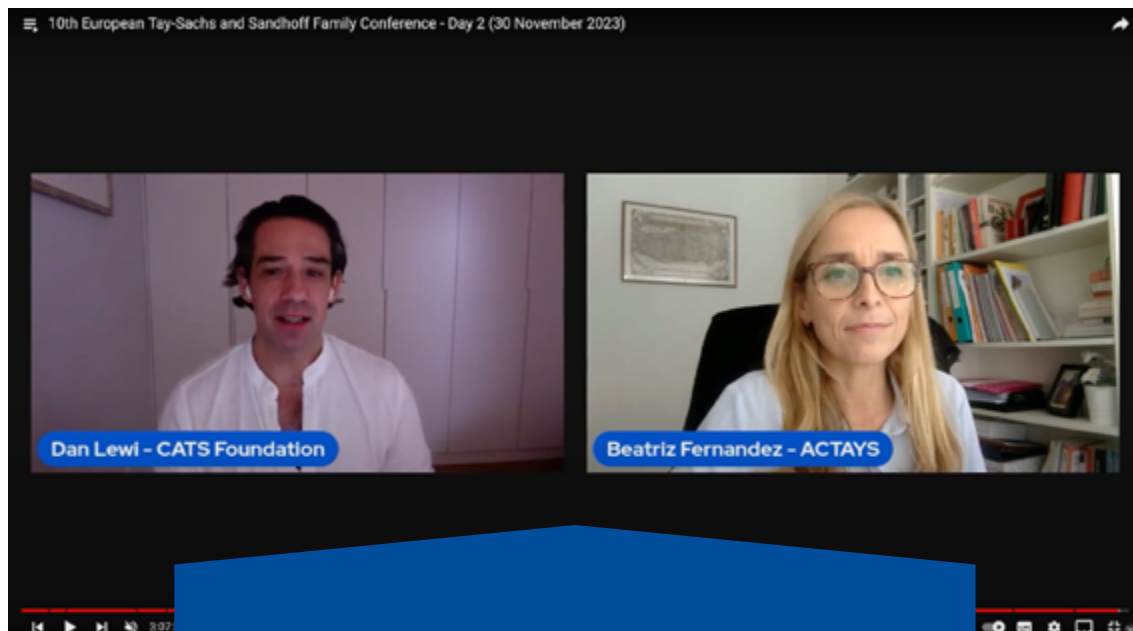
To watch the [presentation click here](#)

# SUMMARY

## DAY 2

A day of updates on the future of treatments for Tay-Sachs and Sandhoff.

It was exciting to hear the updates to the treatments that are being developed for Tay-Sachs and Sandhoff. Not only have many of these moved forward to the next stage, but they are showing a real commitment to the community. It is important for everyone to be kept up to date on this work and one of the core values at the ETSCC is to have transparency on these studies with the Tay-Sachs and Sandhoff community so that they are aware of their progress. It is vital that they continue to be empowered with information and have their questions answered.



[CLICK HERE TO WATCH THE SUMMARY](#)

# ETSCC GOALS FOR 2024

## **1** WORK TOGETHER ON THE DEVELOPMENT OF TREATMENTS FOR OUR COMMUNITY

As a consortium, all member of the ETSCC will continue to work collaboratively for the Tay-Sachs and Sandhoff community so that we help bring treatments to families.

## **2** BECOMING AN ACTIVE MEMBER OF THE NEW GGGG

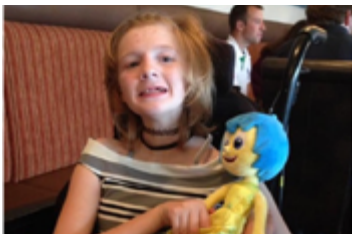
Supporting the global community is a commitment that most members of the ETSCC have made to ensure that the Tay-Sachs and Sandhoff community continues to grow in new regions.

## **3** CONTINUE TO SHARE IMPORTANT INFORMATION

The consortium members plan to continue to share information with families to enhance their collective knowledge and power as members of the Tay-Sachs and Sandhoff community.

## **4** ADVOCATE FOR FAMILIES SO THEY HAVE ACCESS TO ALL THE SUPPORT THEY NEED

Each consortium member will continue to share and advise each other so that we can ensure that families have access to all support they need to manage both now and in the future.







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