

Another year has just begun, and as always when a new year starts, the challenges and goals of the Niemann-Pick Foundation in Spain are renewed.

In this newsletter we are going to explain the latest events and acts that we have prepared and attended since October until today, and the objectives we are working on for the coming months.

October was Niemann-Pick disease awareness month. All countries join forces to make society aware of the disease and commit to the families and organizations that seek to find a cure and offer a better quality of life for those affected. Throughout this month, we carried out and participated in different awareness- raising actions.



On October 16th, we also held the 14th edition of the Toledo Night Race for the benefit of the Foundation, with 4,000 runners, which was a resounding success in terms of participation. We took advantage of the event to publicize and raise awareness about Niemann-Pick in the month of October.

In November, we attended the 1st Congress held by the patient association ASMD Spain in Madrid. There, we were able to listen to professionals who spoke about the latest news about ASMD, historically known as Niemann-Pick type

B. We exchanged words of collaboration with its president, and we were able to greet specialist doctors who are part of our medical committee, such as Dr. Villarrubia or Dr. Ledesma.

On November 30th, we participated in the 16th Valencian Forum on Rare Diseases, organized by FEDER. It was an online event and was attended by our representative in Valencia.

To end the year in style, on 31st December 2021, the San Silvestre Machotera was held in Zarzuela del Monte, Segovia, also for the benefit of the Foundation. The 2021 edition had a total of 317 participants. It was held again after it could not be held last year due to the pandemic.



January 2022 was a busy month. We started by participating in the 1st Catalan Forum on Rare Diseases organized by FEDER. It was an online event in which our family coordinator, Julia González, participated.

Also, in January we started a new initiative at the Foundation: the family of the month. With this initiative we aim to recognize the involvement of families with the Foundation and with all those affected by Niemann-Pick. The first family has been the Moreno Novo family, parents of Adrián, Noa and Daniel. An example for all of us of struggle, empathy, and solidarity. Daniel, from heaven, is very proud of his family!

Also in January, the Foundation's secretary, and Carmelo Fernández, a father of the foundation, met with Juan Carrión, president of FEDER, to personally explain our clinical trial with Efavirenz. We are very hopeful, as you know, about our trial and we have very good news: in a couple of months, in April, we will finally start! We will keep you informed when it has started so that you can know, firsthand, how it is evolving.

In addition, we also participated in the Public Workshop Endpoint Considerations to Facilitate Drug Development for Niemann-Pick Type C (NPC) organized by the Duke Margolis Center for Health Policy and the FDA.

It was two days of learning and receiving information that we are sure we will be able to apply to Niemann-Pick in the future.

February, as all of you in this community know, is a very special month for all of us who live with rare disease patients and their families.

And not only because February 28<sup>th</sup> is International Rare Disease Day, but because on February 17<sup>th</sup> we also commemorated our national day in Spain. This year we commemorated it with a virtual meeting of families on 17<sup>th</sup> February. It was a very special day for us because we all met again, albeit virtually, after many months. And it was very emotional and special for all of us. Hopefully next year we will be able to recover the face-to-face events in towns and cities for this day.



We also participated in three radio interviews, from Segovia, Madrid and Sevilla, to spread and raise awareness about the disease.

The families of the Foundation are going to participate in many of the activities in the framework of World Rare Disease Day. One of them will be that many of us will participate in the virtual race for hope, a virtual race that will





be held between February 28th and March 20th. Many rare disease patient organizations will participate because it is organized by FEDER. We are all going to run for hope; because we are not going to lose hope as long as we still have some way to go.

On February 8th, we also attended the open days organized by FEDER in Valencia, where Carmina, the representative of the Niemann-Pick Foundation in Valencia, was present.

In addition to this, on February 17th, we took part in the World Day for Rare Diseases held in Lleida, organized by the Plataforma de Malalties Minoritàries de Catalunya (Catalan Rare Diseases Platform).

In addition to this day, on February 28th, we will be attending the central event of the Plataforma de Malalties Minoritàries in Barcelona.

On February 23rd we have an event with the La Caixa Foundation, an online debate on why 95% of rare diseases have no treatment.

On February 25th, we participated in the rare disease day organized by CREER and on February 28th, we will also attend the CIBERER event for World Rare Disease Day. In addition, and to end the month of February, we will connect to the global event for Rare Disease Day through EURORDIS.



Finally, we would like to announce that next May on 27-29<sup>th</sup>, we will be holding our 22nd Annual International Scientific-Family Congress in Barcelona. We are already preparing all the logistics and the papers to be presented at the congress. We hope it will be a success and that the pandemic situation will allow us to celebrate it face to face. We will keep you informed to tell you all the news of this congress! We hope some of you could attend in our Congress in Barcelona!

We hope you are all very well.

From Spain... we send you all a big hug!  
See you in the next newsletter!