

# Merry Christmas

from

# ORPHA **Z** YME



## Message from CEO Anders Hinsby

2016 has been an eventful year at Orphazyme, and it's my pleasure to tell you about some of our biggest achievements from the past twelve months. We are extremely proud of the progress we've made in the fight against Niemann-Pick type C and one of the most important moments of 2016 for us was the long-awaited launch of the interventional AIDNPC study. Since the first patient began treatment we have enrolled thirteen patients, which is fantastic!

I'd like to take this opportunity to thank everyone who has helped make this trial a reality, and in particular I'd like to extend my heart-felt gratitude to the young people with Niemann-Pick type C who are participating in this trial. Without your valuable commitment, and the support of your families, we couldn't progress with arimoclomol as a treatment for this devastating disease.

I'm delighted to share with you all my warmest wishes for Christmas and the New Year, and look forward to another year of big steps towards fighting rare diseases.



29,840 km travelled to join family conferences

9 new team members

337 m2 of floor space in our new office

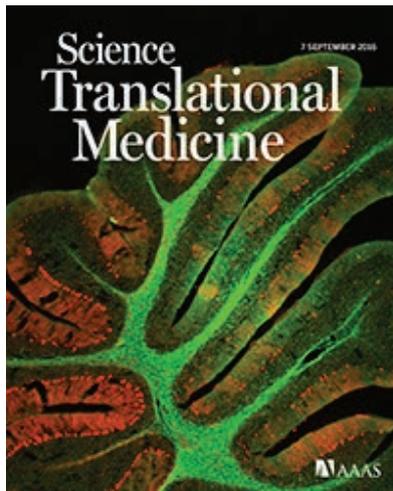
22,400 Twitter impressions

## 2016 at Orphazyme in numbers

**8983** downloads of our latest research publication

Our collaborations with some of the best academic minds have helped us to produce excellent research. This year some of those research efforts were

published in a high impact scientific journal. In fact, the story was featured on the journal's front cover. This research was about the heat shock response as a potential treatment for a range of



lysosomal storage diseases, tested using laboratory models. Our ongoing clinical work is strongly based on the results published here, and this article represents many people's hard work and dedication over many years.

**13** enrolled on the AIDNPC trial

The clinical trial to investigate the experimental drug arimoclomol as a treatment for Niemann-Pick type C got underway this year, and already thirteen young people with Niemann-Pick type C have been enrolled. The study now has patients in Paris, Mainz, Birmingham, Copenhagen and Munich. The trial also expanded to the USA, with sites expected to begin recruiting early in the new year. We are pleased with how the trial is progressing, and we're looking forward to 2017, particularly to the completion of the earlier AIDNPC 'observational' study.



**25** danced to raise awareness of Niemann-Pick & Gaucher diseases

In the Autumn we marked Niemann-Pick and Gaucher disease awareness month by staging a dance flash mob in a Copenhagen shopping centre. Unsuspecting shoppers were saw two dance routines choreographed by professional dancers. We certainly had



a lot of fun performing, and we're already thinking creatively about how we can mark occasion next year, and raise awareness of these diseases.

**Keep in touch**

Contact us by email on [contact@orphazyme.com](mailto:contact@orphazyme.com) or follow our progress on Twitter [@Orphazyme](https://twitter.com/Orphazyme)