

## Clinical Trials Statement – June 2016

**There are two clinical trials for NP-C, and two for ASMD (NP-B) recruiting at present.**

The last clinical trial for NPD, for Miglustat, was almost 10 years ago. Opportunities for finding these long hoped for treatments don't come along very often, and now we have four!

This really is an important time for the Niemann-Pick community. Many individuals and groups, over many years, raising countless millions, have worked towards finding effective treatments for these diseases. None of these trials would be happening without the huge contributions and sacrifices of many families and supporters.

Patient participation in clinical trials is vital and will greatly help to further understanding of potential therapies and whether they work.

The INPDA recognises that every family situation is different and every family must make their own decision.

Not everyone will be eligible to participate in these trials, and for those who are, a clinical trial may not be the right thing.

However, we urge everyone to find out about these trials – to ask the questions you need to, to speak to your doctor, to your patient support group – and to make the best decision, whatever that might be, for you and your family.

### **Trial information:**

**The Orphazyme AIDNPC clinical trial programme** is recruiting patients to their prospective observational study ahead of intervention with the potential therapy Arimoclomol. Sites in the UK, Denmark, Germany, Italy, Poland, Spain and Switzerland are now recruiting patients (ages 2 – 18 years plus 11 months), with more sites to follow. For further, up-to date information, visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) searching with identifier NCT02435030. Or visit the sponsor's website at [www.aidnpc.com](http://www.aidnpc.com).

**Vtesse Phase 2b/3 clinical trial of VTS-270** (2-hydroxypropyl- $\beta$ -cyclodextrin) to treat Niemann-Pick disease Type C1 (NP-C1) disease is now recruiting patients at sites in the UK, USA and Spain. Sites in Germany and France are active but not currently recruiting. The age range eligible for the study has been widened to include those 4 to 21 years. For further, up-to date information, visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) searching with identifier NCT02534844. Or visit the sponsor's website at [www.thenpcstudy.com](http://www.thenpcstudy.com)

**Sanofi Genzyme** is recruiting adult (18 years and older) patients to a study of “Olipudase Alfa” to treat patients with acid sphingomyelinase deficiency, ASMD (Niemann-Pick B). Sites in France, Germany, Netherlands, Turkey, Japan and UK are currently recruiting with more sites to follow. For further, up-to date, information, visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) searching with identifier NCT 02004691.

**Sanofi Genzyme** is recruiting paediatric patients (below 18yrs) at sites in UK, USA, Germany, Brazil and Italy to a study of “Olipudase Alfa” to treat patients with acid sphingomyelinase deficiency, ASMD (Niemann-Pick B). For further information, visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) searching with identifier NCT02292654