

NIEMANN-PICK TYPE C DISEASE (NP-C) IS A

LONG RARE PROGRESSIVE IRREVERSIBLE CHRONICALLY DEBILITATING LYSOSOMAL STORAGE DISEASE¹⁻³

NP-C affects all ages¹



Incidence of NP-C is 1 in 90,000 live births⁴

Likely an underestimate due to lack of clinical awareness¹

NP-C takes on average 5 YEARS to diagnose⁵

That's...

1,826 DAYS

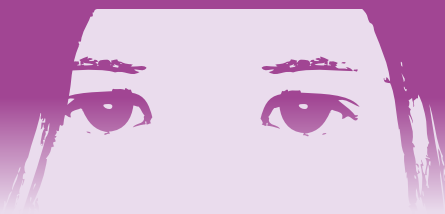
260 WEEKS



43,824 HOURS

...waiting for an answer, watching a loved one get worse

Have you checked for eye movement abnormalities?



Vertical supranuclear gaze palsy (VSGP) is present in virtually all patients^{1,3}

**THINK AGAIN
THINK NP-C**

THINK AGAIN. THINK NP-C aims to support healthcare professionals unfamiliar with NP-C to recognise the key signs and symptoms of NP-C and reduce the time to diagnosis

Individual symptoms are non-specific to the disease^{1,3}

If you are a: **Paediatrician**

LOOK FOR ATAXIA, DEVELOPMENTAL DELAY, HEPATOSPLENOMEGALY



Paediatric hepatologist/neonatologist

LOOK FOR HEPATO/SPLENOMEGALY, NEONATAL CHOLESTATIC JAUNDICE, NEONATAL LIVER DYSFUNCTION

Adult neurologist/psychiatrist

LOOK FOR COGNITIVE DECLINE, ORGANIC PSYCHOSIS, PROGRESSIVE ATAXIA



References

1. Patterson M, Hendriksz, Walterfang M, et al. on behalf of the NP-C Guidelines Working Group. Recommendations for the diagnosis and management of Niemann-Pick disease type C: an update. *Mol Genet Metab* 2012; 106(3): 330–344.
2. Vanier, M. Niemann-Pick disease type C. *Orphanet J Rare Dis* 2010; 5: 16.
3. Wijburg FA, Sedel F, Pineda M, et al. Development of a suspicion index to aid diagnosis of Niemann-Pick disease type C. *Neurology* 2012;78(20):1560–1567.
4. Wassif C, Cross J, Iben J et al. High incidence of unrecognized visceral/neurological late-onset Niemann-Pick disease, type C1, predicted by analysis of massively parallel sequencing data sets. *Genet Med* 2016; 18(1): 41–48
5. Mengel E, Klünemann H, Lourenço C, et al. Niemann-Pick disease type C symptomatology: an expert-based clinical description. *Orphanet J Rare Dis* 2013; 8: 166.

To help reduce the time to diagnosis visit www.think-npc.com today

**THINK AGAIN
THINK NP-C**

This is a project co-ordinated by the International Niemann-Pick Disease Alliance with the collaboration and financial support of Actelion Pharmaceuticals Ltd

PROGRESS TOGETHER
Inpoda
International Niemann-Pick Disease Alliance