

Targeting sphingosine storage as a new therapeutic approach for Niemann-Pick Type C

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Lay Summary – Update December 2010

We have been evaluating the combination of miglustat and curcumin in the NPC1 mouse and have found that this achieves considerable benefit, in terms of reducing symptoms and prolonging life span. To date it achieves the greatest benefit of any drug/drug combination that we have tested.

We have also established an assay for measuring lipids in these mice, including sphingosine. Tissues from the combination and single therapy treatment groups are under biochemical analysis and will be completed early in 2011.

In summary, this grant has validated the benefit of combination therapy of miglustat and curcumin in NPC mice. We are indebted to NNPfF for their support.

