

Reviewer's report

Title: Observational cohort study of the natural history of Niemann-Pick disease type C in the UK: a 5-year update from the UK clinical database

Version: 0 Date: 21 Oct 2015

Reviewer: Kuo-Hsuan Chang

Reviewer's report:

This manuscript by Imrie and colleagues updated the observational study of the natural history of Niemann-Pick disease type C in the UK. This manuscript is of good quality, as well as important for the clinical description of this rare disease. A comparison of clinical features/severity of 52 newly diagnosed patients to 94 previously reported ones may be helpful to understand the temporal improvement of disease diagnosis and awareness.

Are the methods appropriate and well described?

If not, please specify what is required in your comments to the authors.

Yes

Does the work include the necessary controls?

If not, please specify which controls are required in your comments to the authors.

Unable to assess

Are the conclusions drawn adequately supported by the data shown?

If not, please explain in your comments to the authors.

Yes

Are you able to assess any statistics in the manuscript or would you recommend an additional statistical review?

If an additional statistical review is recommended, please specify what aspects require further assessment in your comments to the editors.

I am able to assess the statistics

Quality of written English

Please indicate the quality of language in the manuscript:

Acceptable

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