


CORRECTION

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Correction to: Clinical disease progression and biomarkers in Niemann–Pick disease type C: a prospective cohort study

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Correction to:

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Following the publication of the original article [1], it was brought to the authors' attention that there are several errors within the article, introduced through both

typographical and editorial oversight as well as through final typesetting.

The errors and their respective corrections are shown in Table 1. The authors apologise for these errors, but note that the results and conclusions of the manuscript remain unchanged.

The original article can be found online at <https://doi.org/10.1186/s13023-020-01616-0>.

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Table 1

Error	Correction
Abstract background, page 1: cholestane-3 β ,5 α -,6 β -triol	cholestane-3 β ,5 α ,6 β -triol
<i>Abstract results, page 1, lines 4-7:</i> Compared with healthy individuals, the NPC population had significantly lower levels of cholesterol esterification ($p < 0.0001$), HSP70 ($p < 0.0001$) and skin unesterified cholesterol ($p = 0.0006$). Cholestane-triol levels were significantly higher in individuals with NPC versus healthy individuals ($p = 0.008$) and correlated with the 5-domain NPCCSS (Spearman's correlation coefficient = 0.265, $p = 0.0411$).	Compared with healthy individuals, the NPC population had significantly lower levels of cholesterol esterification ($p < 0.0001$) and HSP70 ($p < 0.0001$), and significantly higher levels of skin unesterified cholesterol ($p = 0.0006$). Cholestane-triol levels were also significantly higher in individuals with NPC versus healthy individuals ($p = 0.008$) and were correlated with the 5-domain NPCCSS (Spearman's correlation coefficient = 0.265, $p = 0.0411$).
<i>Abstract trial registration, page 2:</i> 2014-005,194-37	2014-005193-37
<i>Background, page 2, paragraph 2, line 9:</i> that affect gross motor skills, swallowing ability cogni-	tion still had an end of study visit and provided data for
<i>Results, page 4, paragraph 4, line 11:</i> tion still had an end-of-trial visit and provided data for	by referring clinicians as current medical conditions, were
<i>Results, page 4, paragraph 6, line 3:</i> by referring clinicians as current medical conditions, was	(Fig. 3c).
<i>Results, page 4, paragraph 7, line 9:</i> (Fig. 3b).	an end of study visit and is included in efficacy assessments
<i>Figure 3 legend, line 4,</i> an end-of-trial visit and is included in efficacy assessments	Total number of distinct alleles
<i>Table 3, row 1:</i> Total number of different alleles	Disease biomarkers over the 6–14-month observation period and those of healthy individuals
<i>Table 4, title:</i> Change in disease biomarkers over the 6–14-month observation period compared with those of healthy individuals	(Table 7).
<i>Results, page 8, paragraph 4, line 20:</i> (Table 6).	a. Skin unesterified cholesterol. b. PBMC unesterified cholesterol.
<i>Figure 5 legend, line 1-2:</i> a. PBMC unesterified cholesterol. b. Skin unesterified cholesterol.	This is incorrectly indented, please align to the left.
<i>Table 5, line 20:</i> Serious adverse events [indented]	Table 7
Table 6	Table 6
Table 7	(Table 6.)
<i>Results, page 11, paragraph 2, line 12:</i> (Table 7).	Mean 5-domain NPCCSS score (\pm SD)
<i>Table 7, heading:</i> MEAN 5-domain NPCCSS score (\pm SD)	date and to confirm the patients' and clinicians' views on clinically meaningful changes on the 5-domain NPCCSS
<i>Discussion, page 12, paragraph 1, line 12-13:</i> date and to confirm the patients and clinicians view on clinical meaningful changes on the 5-domain NPCCSS	tane triol burden with NPC disease severity [22]. However,
<i>Discussion, page 13, paragraph 2, line 12:</i> tane burden with NPC disease severity [22]. However,	this was likely due to the limited viability of PBMCs
<i>Discussion, page 13, paragraph 3, line 19:</i> this was likely owing to the limited viability of PBMCs	uting to the 5-domain NPCCSS was locally independent
<i>Discussion, page 14, paragraph 3, line 11:</i> uting to the 5-domain NPCCSS were locally independent	Delete
<i>Methods, page 15, paragraph 2, line 9:</i> Incorrect formatting of citation (15)	(ICC) [2,13]
<i>Methods, page 16, paragraph 6, line 22:</i> Incorrect formatting of abbreviation and citation (ICC2,13)	Delete
<i>Abbreviations:</i> MCID: Minimal clinically important difference;	

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1. Mengel E, et al. Clinical disease progression and biomarkers in Niemann-Pick disease type C: a prospective cohort study. *Orphanet J Rare Dis*. 2020;15:328. <https://doi.org/10.1186/s13023-020-01616-0>.

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