**Reviewer’s report**

**Title:** Treatment with PBI-4050 in patients with Alström syndrome: study protocol for a Phase 2, single-centre, single-arm, open-label trial

**Version:** 1  **Date:** 25 Jul 2018

**Reviewer:** Gabriella Milan

**Reviewer's report:**

This article explains the treatment trial of patient with Alstrom syndrome with PIB-4050 an anti-fibrotic and anti-inflammatory agent.

This drug was previously tested on preclinical models and patients with metabolic syndrome and idiopathic pulmonary fibrosis. PIB-4050 may have interesting effects on Alstrom syndrome patients because of their multiorgan fibrosis.

Despite the low number of patients enrolled, it is important to let know this possibility for patients that had not other therapeutic options.

This study was well designed and provided sufficient details to allow replications and comparisons; statistical analysis is appropriate and there is no problem on writing.

I have only few comments:

Page 14 line 1, specify ELF test acronym

Page 18 line 36, probably "if" not "is".
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.

Yes

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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