ESPGHAN 56th Annual Meeting: The 56th Annual Meeting of the European Society for Paediatric Gastroenterology, Hepatology, and Nutrition

Meeting dates: 15−18 May 2024

Meeting location: Milan, Italy

Abstract submission deadline: Monday, 11 December 2023: Midnight CET (23:59)

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SUMMARY OF SOCIETY'S GUIDELINES FOR AUTHORS

Link to Author Guidelines: https://espghancongress.org/abstract-submission-guidelines/

- Word count limit: 300 words including acknowledgements; (Note: word count is affected when graphs/tables are included).
- Abstracts must contain data and meet international ethical standards
- Abbreviations should be defined
- Use generic names of drugs
- One table may be included with your submission: Max number of columns: 10; Max number of rows: 10
- Please note that a table may significantly reduce the number of available words; Instead of the table you can include an image: The maximum file size of each image is 500 KB. The maximum pixel size of the graph/image is 600(w) x 800(h) pixel. You may upload images in JPG, GIF or PNG format. Please Note: images do not affect the word count
- Submissions may not contain patient names, hospital ID numbers or other identifying information
- Must select a specific Theme, Topic and/or Sub-Topic; Abstract topic– select the abstract topic per the list of topics
- The presenting author must be a registered participant
- The same person may submit as many abstracts as they would like
- The same person may serve as a presenting author on up to 5 abstracts

EFFICACY AND SAFETY OF ODEVIXIBAT OVER 48 WEEKS: POOLED DATA FROM THE PHASE 3 ASSERT AND ASSERT-EXT STUDIES IN PATIENTS WITH ALAGILLE SYNDROME

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ABSTRACT:

Objectives and Study: Cholestasis in Alagille syndrome (ALGS) is associated with bile acid (BA) accumulation in the liver with spill-over into the systemic circulation, as well as severe pruritus that can impair sleep. Here, we describe outcomes with odevixibat, an ileal BA transporter inhibitor, using pooled data from the phase 3 ASSERT and ASSERT-EXT trials in patients with ALGS.

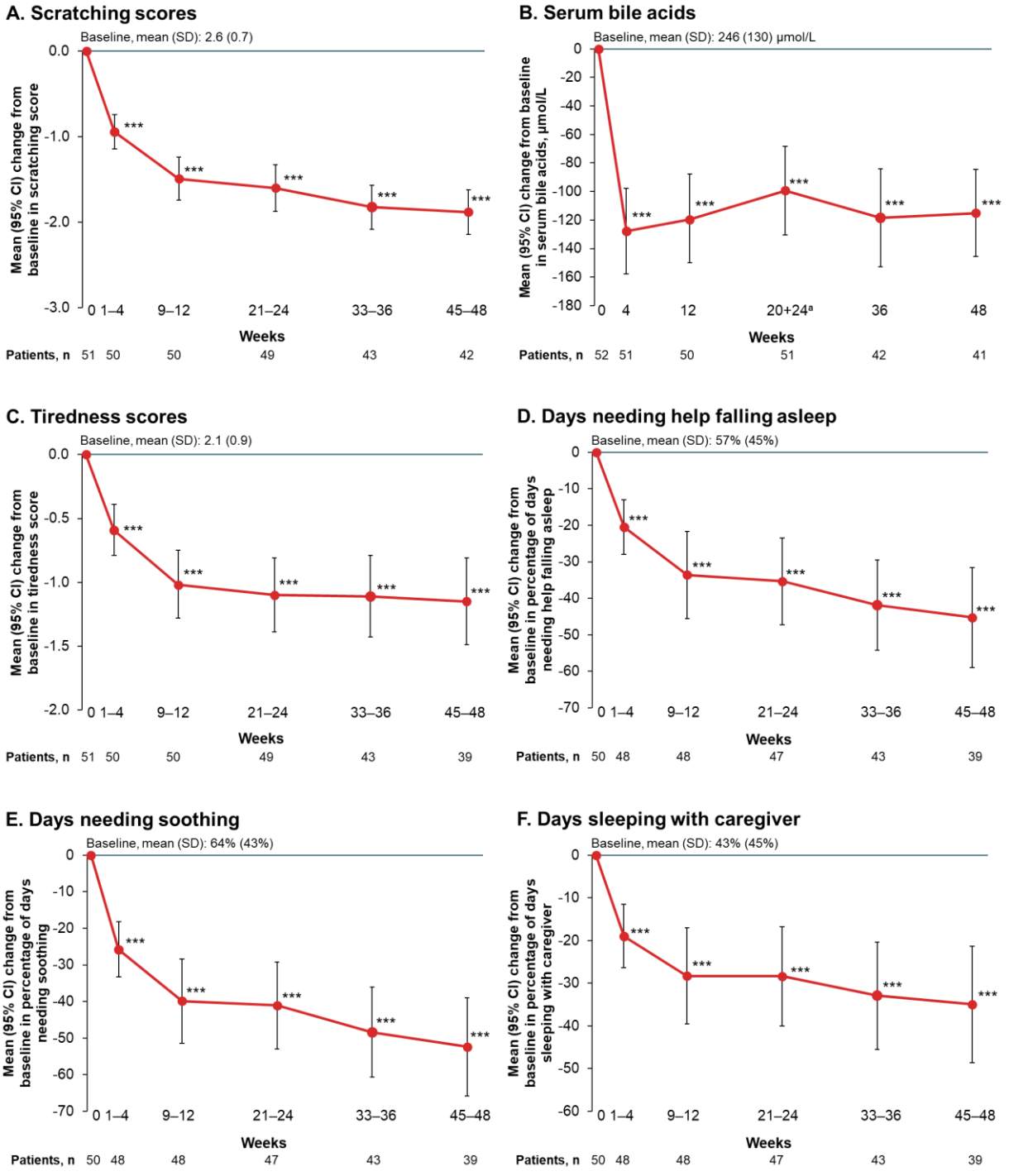
Methods: In ASSERT (NCT04674761), patients with ALGS with history of significant pruritus and elevated serum BAs (sBAs) were randomised 2:1 to odevixibat 120 µg/kg/day or placebo, respectively, for 24 weeks. Patients who completed ASSERT could enter ASSERT-EXT (NCT05035030), an ongoing, 72-week extension study

where all patients received odevixibat 120 µg/kg/day. Here, data from odevixibat-treated patients in ASSERT and/or ASSERT-EXT were pooled (from first odevixibat dose to a data cut-off of 17 July 2023). Observer-reported scratching scores, sleep parameters, and sBA levels were assessed through 48 weeks of treatment.

Results: At the data cut-off, the pooled population comprised 52 odevixibat-treated patients (mean age, 6.5 years; 48% female; median [range] odevixibat exposure, 73 [16–110] weeks). Odevixibat treatment for up to 48 weeks resulted in rapid and significant mean improvements in pruritus and reductions in sBA levels vs baseline (**Figure**). There were also significant decreases from baseline to weeks 45−48 in multiple sleep parameters, including tiredness and mean percentage of days patients needed help falling asleep, needed soothing, and slept with their caregiver (**Figure**). Treatment-emergent adverse events (TEAEs) were reported in 49 of 52 (94%) odevixibat-treated patients. The most common TEAE was diarrhoea (n=18/52 [35%]). At the data cut-off, 1 patient had a TEAE (blood bilirubin increased) that led to study discontinuation.

Conclusions: In patients with ALGS, odevixibat treatment for up to 48 weeks led to significant improvements in pruritus and sleep and significant reductions in sBA levels. TEAEs in this pooled analysis were consistent with previously reported results.

Figure. Mean change from baseline in scratching scores (A), serum bile acids (B), and sleep parameters (C−F) in a pooled population of patients with Alagille syndrome treated with odevixibat



Scratching and tiredness scores were measured using the PRUCISION observer-reported outcome instrument (range, 0−4; higher scores indicate worse symptoms). [a]Average of weeks 20 and 24. ****P*<0.001.

Main topic: AS02. HEPATOLOGY

Sub-topic: AS02a. General Hepatology

Presentation preference: Oral presentation

Keywords: Alagille syndrome, pruritus, ileal bile acid transporter inhibitor

Conflicts of Interest

N. Ovchinsky: Albireo, an Ipsen Company – Consultant; Albireo, an Ipsen Company, Mirum, and Travere – Research support
M. Aumar, P. Czubkowski, Ö. Durmaz, and **W. S. Lee:** Nothing to disclose
A. Baker: Albireo, an Ipsen Company, and Mirum – Research support
U. Baumann: Albireo, an Ipsen Company, Mirum, Alnylam, Vivet, and Nestlé – Consultant
P. Bufler: Albireo, an Ipsen Company, Mirum, Orphalan, Nestlé Nutrition Institute, Nutricia, Alexion, Univar, Amgen, and AbbVie – Consultant and/or lecture fee
M. Cananzi: Albireo, an Ipsen Company, Mirum, CTRS, and Nestlé – Consultant
R. Fischer: Albireo, an Ipsen Company, and Mirum – Consultant and/or speaker
G. Indolfi: Albireo, an Ipsen Company, Mirum, and Kedrion Pharma – DSMB and/or advisory board participant
W. W. Karnsakul: Albireo, an Ipsen Company, Travere, Mirum, Gilead, and AbbVie – Research support and consultant
F. Lacaille: Alexion – Consultant
G. Maggiore: Albireo, an Ipsen Company, Mirum, Alexion, and Orphalan – Consultant
P. Rosenthal: AbbVie, Albireo, an Ipsen Company, Arrowhead, Gilead, Merck, Mirum, Takeda, and Travere – Research support; Albireo, an Ipsen Company, Ambys, Audentes, BioMarin, Dicerna, Encoded, Gilead, MedinCell, Mirum, Takeda, and Travere – Consultant
M. Ruiz: Albireo, an Ipsen Company, Grifols, Mirum, and Takeda – Consultant
E. Sokal: Albireo, an Ipsen Company – Consultant and investigator; Mirum and Intercept – Investigator; Cellaion – Chairman and CEO

E. Sturm: Albireo, an Ipsen Company, and Mirum – Consultant and research support; Univar – Consultant; Orphalan – Speaker's fee

W. van der Woerd: Mirum – Consultant

H. J. Verkade: Ausnutria BV, Albireo, an Ipsen Company, Danone Nutricia Research, Intercept, Mirum, Orphalan, and Vivet – Consultant

A. Wehrman: Albireo – Research support; Mirum – Advisory board

J. Ruvido, Q. Ni, and **S. Manganaro:** Albireo, an Ipsen Company – Employment