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SBI Pharmaceuticals Co., Ltd.

Publication of Investigator-initiated Clinical Trials of 5-ALA Hydrochloride and Sodium Ferrous Citrate for Pediatric Patients with Mitochondrial Disease

SBI Pharmaceuticals Co., Ltd., (Head office: Minato-ku, Tokyo; Representative Director & President: Yoshitaka Kitao, hereinafter "SBI Pharma"), a subsidiary of SBI Holdings, Inc., engaged in the research and development of pharmaceuticals and medical devices regarding 5-aminolevulinic acid (5-ALA) *, hereby announces the publication of the results of investigator-initiated clinical trials of SPP-004 (5-Aminolevulinic acid hydrochloride and sodium ferrous citrate) for pediatric patients with mitochondrial disease, which were supported with SBI Pharma, in Life, an international scientific journal on life sciences.

Journal	Life
Title	Efficacy and Safety of 5-Aminolevulinic Acid Hydrochloride Combined with Sodium Ferrous Citrate in Pediatric Patients with Leigh Syndrome and Central Nervous System Disorders: An Initial Exploratory Trial with a Double-Blind Placebo-Controlled Period, Followed by an Open-Label Period and a Subsequent Long-Term Administration Study
Authors	Yuichi Abe (Department of Pediatrics, Saitama Medical University , Present address: Division of Neurology, National Center for Child Health and Development) et al (Corresponding author: Akira Ohtake).
URL	https://www.mdpi.com/2075-1729/15/8/1168
Abstract	The safety and efficacy of SPP-004 (5-ALA hydrochloride and SFC) was evaluated in 10 patients under the age of 2 with Leigh syndrome, one of the major pediatric mitochondrial diseases. An exploratory study (SPED-ALA-001 study) consisting of a 12-week double-blind period receiving SPP-004 or Placebo and a 12-week open-label period receiving SPP-004, and a subsequent long-term administration study (SPED-ALA-002 study) in which SPP-004 was administered continuously for

	up to 180 weeks were conducted. There was no significant difference in NPMDS scores (criterion for assessing the severity of mitochondrial disease), between the SPP-004 and placebo groups at the end of the double-blind period, but the symptoms suggested to be stabilized or improved with subsequent long-term administration of SPP-004 from the open-label period until the end of the long-term administration study. Blood lactate levels increased in the placebo group during the double-blind period, but the average level was normalized with long-term administration of SPP-004. One patient died of heart failure presumably due to underlying disease, but there were few serious adverse drug effects, and seven patients safely completed long-term treatment. These results suggest that SPP-004 may contribute to stabilization of symptoms in pediatric patients with mitochondrial disease.

*1 5-aminolevulinic acid: An amino acid produced in mitochondria. It is an important substance that serves as a functional molecule related to energy production in the form of heme and cytochromes, and its productivity is known to decrease with age. 5-aminolevulinic acid is contained in food such as shochu lees, red wine, and Asian ginseng. It is also known as a material forming chloroplasts in plants.

*2 This new release is an introduction to the research publication. It does not recommend the use of unapproved drugs.

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For further information:
SBI Pharmaceuticals Co., Ltd.
E-mail: info_ala@sbigroup.co.jp