**Background**

- In Europe, growth hormone (GH) therapy is indicated for use in children born small for gestational age (SGA) who fail to demonstrate adequate catch-up growth. However, treatment should be initiated no earlier than 4 years of age, as opposed to 2 years of age in the United States. 2

- Induction of treatment at younger age and use of higher doses are predictors of successful early growth in short-stature children born SGA who are treated with GH therapy. 3

- Being born SGA is associated with subtle impairments in cognitive performance and educational achievement. 4 Studies conducted to investigate the potential beneficial effect of GH therapy on cognitive development in short-stature children aged 3–11 years who were treated for at least 2 years have yielded mixed results. 5

- Only few studies have been conducted to assess the efficacy and safety of GH treatment in very young (<10 months) short-stature children born SGA, starting at the age of 24 to 30 months.

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**Methods**

- Randomization

- Controls to treatment

- Treatment

- Follow-up

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**Assessments**

- Standing height, body weight, BMI, head circumference, and palmar status were assessed at study entry and at 6, 12, 18, and 24 months. SDS values were calculated for height and weight using the method of Greulich and Pyle.

- Assessment of psychomotor development using the Bayley Scales of Infant Development, 2nd edition (BSID-II) was performed at study entry and at 24 months.

- An o-ray of the head and wrist was performed at study entry and at 12 and 24 months to permit assessment of bone age using the method of Greulich and Pyle.

- All observed or volunteered adverse events (AEs) were reported.

- Blood samples were taken for measuring fasting blood glucose, insulin-like growth factor-1 (IGF-1), insulin-like growth factor binding protein-3 (IGFBP-3), thyroxine (T4), thyroid stimulating hormone (TSH), alkaline phosphatase, platelets, and leucocytes, haematocrit, alanine aminotransferase, albumin, sodium, potassium, calcium, urea, and creatinine.

- Treatment-emergent adverse events (TEAEs) were defined as any adverse event that developed or worsened during the treatment period or within 30 days after the last dose of study medication.

**Statistical analysis**

- Change in height SDS at 12 and 24 months was analysed using LOCF data in an ANCOVA, with baseline height SDS and treatment as covariates.

- Change in growth velocity SDS at 12 and 24 months was analysed using LOCF data in an ANCOVA, with baseline growth velocity SDS and treatment as covariates.

- Change in MAD and IFD of the SDS-II was also analysed at 12 months using observed data in an ANCOVA, with baseline age, gender, and treatment as covariates.

**Results**

- In total, 52 subjects from 13 centres from 8 European countries were screened for the study.

- 45 children (21 in Genotropin arm [11 male, 12 female], 22 in control arm [12 male, 11 female]), aged 10–29 months, and presenting a height below –2.5 SD at screening received treatment (Figure 1). Genotropin dose adjustment was performed at each visit.

- The primary endpoint was change in baseline SDS at 24 months of treatment with Genotropin.

**Conclusions**

- In very young, short-stature (height SDS below –2.5) children who were born SGA and who failed to show early catch-up growth, an approximately 2 years of age, administration of Genotropin therapy at a dose of 0.05 mg/kg/day for 24 months resulted in significant growth recovery as measured from baseline in height SDS.

- A significant increase in head growth occurred during Genotropin treatment, no significant change in psychomotor and mental development was observed, although the study was not powered on this endpoint.

- Genotropin therapy was well tolerated in very young children born SGA. The AEs observed in this study were consistent with the safety profile of GH therapy and the background occurrence of common childhood infections in this age group.

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**References**

1. LBP-D3-1005


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