A New Era in Relieving the Burden and Improving Health Outcomes of Children with **Growth Hormone Deficiency**

Clinical Resources

ClinicalTrials.gov (https://www.clinicaltrials.gov)

- NCT03972345: Influence of adherence to growth hormone therapy (GHT) with Norditropin on near final height in patients with growth hormone deficiency (GHD) and born small for gestational age (SGA)
- NCT03309891: Dose finding study of GX-H9 in paediatric patients with growth hormone deficiency
- NCT04326374: Safety, tolerability and efficacy of TransCon hGH weekly versus daily hGH in Chinese pediatric growth hormone deficiency
- NCT04614337: Phase 2 study of LUM-201 in children with growth hormone deficiency (OraGrowtH210 Trial)
- NCT04633057: A study to evaluate the efficacy and safety of recombinant long-acting human growth hormone (TJ101) in children with growth hormone deficiency
- NCT02968004: Safety and efficacy phase 3 study of long-acting hGH (MOD-4023) in growth hormone deficient children
- NCT03831880: Patient perception of treatment burden in weekly versus daily growth hormone injections in children with GHD.
- NCT04513171: Safety and efficacy of Y-shape pegylated somatropin in growth hormone deficiency children
- NCT03290235: Extension study of pegylated somatropin to treat growth retardation caused by endogenous growth hormone deficiency in children
- NCT04970654: A research study in Chinese children with a low level of hormone to grow. Treatment is somapacitan once a week compared to Norditropin once a day.
- NCT03811535: A research study in children with a low level of hormone to grow. Treatment is somapacitan once a week compared to Norditropin once a day (REAL4)

Medline Plus: https://medlineplus.gov/growthdisorders.html

Merck Manual: https://www.merckmanuals.com/professional/pediatrics/endocrine-disorders-in-children/growth-hormone-deficiency-in-children

National Organization for Rare Disorders: https://rarediseases.org/rare-diseases/growth-hormone-deficiency/

Guidelines

American Academy of Pediatrics. Braun LR, Marino R. Disorders of growth and stature. *Pediatr Rev*. 2017;38(7):293-304. doi:10.1542/pir.2016-0178

American Association of Clinical Endocrinologists and American College of Endocrinology. Yuen KC, Biller BMK, Radovick S, et al. Guidelines for management of growth hormone deficiency in adults and patients transitioning from pediatric to adult care. *Endocr Pract*. 2019;25(11):1191-1232. doi:10.4158/GL-2019-0405

American Association of Clinical Endocrinologists. Cook DM, Yuen KCJ, Biller BMK, et al; American Association of Clinical Endocrinologists. American Association of Clinical Endocrinologists medical guidelines for clinical practice for growth hormone use in growth hormone-deficient adults and transition patients – 2009 update. *Endocr Pract*. 2009;15(suppl 2):1-29. doi:10.4158/EP.15.S2.1

American Family Physician. Barstow C, Rerucha C. Evaluation of short and tall stature in children. *Am Fam Physician*. 2015;92(1):43-50.

Pediatric Endocrine Society. Grimberg A, DiVall SA, Polychronakos C, et al; Drug and Therapeutics Committee and Ethics Committee of the Pediatric Endocrine Society. Guidelines for growth hormone and insulin-like growth factor-I treatment in children and adolescents: growth hormone deficiency, idiopathic short stature, and primary insulin-like growth factor-I deficiency. *Horm Res Paediatr*. 2016;86(6):361-397. doi:10.1159/000452150

Clinical Trials and Other Articles

Battelino T, Rasmussen MH, De Schepper J, et al; NN8640-4042 Study Group. Somapacitan, a once-weekly reversible albumin-binding GH derivative, in children with GH deficiency: a randomized dose-escalation trial. *Clin Endocrinol (Oxf)*. 2017;87(4):350-358. doi:10.1111/cen.13409

Briceno LGG, Viaud M, Beltrand J, et al. Improved general and height-specific quality of life in children with short stature after 1 year on growth hormone. *J Clin Endocrinol Metab*. 2019;104(6):2103-2111. doi:10.1210.jc.2018-02523

Brod M, Alolga SL, Beck JF, et al. Understanding burden of illness for child growth hormone deficiency. *Qual Life Res*. 2017;26(7):1673-1686. doi:10.1007/s11136-017-1529-1

Bullinger M, Koltowska-Häggström M, Sandberg DE, Chaplin JE, et al. Health-related quality of life of children and adolescents with growth hormone deficiency or idiopathic short stature – Part 2: Available results and future directions. *Horm Res Paediatr*. 2009;72(2):74-81. doi:10.1159/000232159

Bullinger M, Sommer R, Pleil A, et al. Evaluation of the American-English Quality of Life in Short Stature Youth (QoLISSY) questionnaire in the United States. *Health Qual Life Outcomes*. 2015;13:43. doi:10.1186/s12955-015-0236-2

Deal CL, Pastrak A, Silverman LA, Valluri SR, Wajnrajch MP, Cara JF. Somatrogon growth hormone in the treatment of pediatric growth hormone deficiency: Results of the pivotal pediatric phase 3 clinical trial. *J Endocr Soc*. 2020;4(Suppl):A648-A649. doi:10.1210/jendso/bvaa046.1279

Giesler A, Lass N, Reinsch N, et al. Quality of life in children and adolescents with growth hormone deficiency: association with growth hormone treatment. *Horm Res Paediatr*. 2012;78(2):94-99. doi:10.1159/000341151

Grimberg A, Feudtner C, Gordon CM. Consequences of brand switches during the course of pediatric growth hormone treatment. *Endocr Pract*. 2012;18(3):307-316. doi:10.4158/EP11217.OR

Horikawa R, Tanaka T, Hasegawa Y, et al. Phase 3 study evaluating once weekly somatrogon compared to daily genotropin in Japanese patients with pediatric growth hormone deficiency (pGHD). *J Endocr Soc*. 2021;5(Suppl 1):A682-A683.

Juul Kildemoes R, Rasmussen MH, Agersø H, Overgaard RV. Optimal monitoring of weekly IGF-I levels during growth hormone therapy with once-weekly somapacitan. *J Clin Endocrinol Metab*. 2021;106(2):567-576. doi:10.1210/clinem/dgaa775

Maniatis AK, Caselia SJ, Nadgir UM, et al. Efficacy and safety of up to 2 years of treatment with TransCon hGH (lonapegsomatropin) in treatment-naïve and treatment-experienced children with growth hormone deficiency. *J Endocr Soc*. 2021;5(Suppl 1):A676. doi:10.1210/jendso/bvab048.1378

Ramirez-Andersen HS, Behrens C, Buchardt J, et al. Long-acting human growth hormone analogue by noncovalent albumin binding. *Bioconjug Chem*. 2018;29(9):3129-3143. doi:10.1021/acs.bioconjchem.8b00463

Rasmussen MH, Olsen MWB, Alifrangis L, Klim S, Suntum M. A reversible albumin-binding growth hormone derivative is well tolerated and possesses a potential once-weekly treatment profile. *J Clin Endocrinol Metab*. 2014;99(10):E1819-1829. doi:10.1210/jc.2014-1702

Rosenfeld RG, Bakker B. Compliance and persistence in pediatric and adult patients receiving growth hormone therapy. *Endocr Pract*. 2008;14(2):143–154. doi:10.4158/EP.14.2.143

Sandberg DE, Gardner M. Short stature: is it a psychosocial problem and does changing height matter? *Pediatr Clin North Am*. 2015;62(4):963-982. doi:10.1016/j.pcl.2015.04.009

Sävendahl L, Battelino T, Brod M, et al. Once-weekly somapacitan vs daily GH in children with GH deficiency: Results from a randomized phase 2 trial. *J Clin Endocrinol Metab*. 2020;105(4):e1847-e1861. doi:10.1210/clinem/dgz310

Stanley T. Diagnosis of growth hormone deficiency in childhood. *Curr Opin Endocrinol Diabetes Obes*. 2012;19(1):47-52. doi:10.1097/MED.0b013e32834ec952

Thornton PS, Maniatis AK, Aghajanova E, et al. Weekly lonapegsomatropin in treatment-naïve children with growth hormone deficiency: the phase 3 heiGHt trial. *J Clin Endocrinol Metabol*. 2021;106(11):3184-3195.

Thygesen P, Andersen HS, Behrens C, et al. Nonclinical pharmacokinetic and pharmacodynamic characterization of somapacitan: a reversible non-covalent albumin-binding growth hormone. *Growth Horm IGF Res*. 2017;35:8-16. doi:10.1016/j.ghir.2017.05.006

Yuen KCJ, Alter CA, Miller BS, et al. Adult growth hormone deficiency: optimizing transition of care from pediatric to adult services. *Growth Horm IGF Res*. 2021;56:101375. doi:10.1016/j.ghir.2020.101375

Yuen KCJ, Miller BS, Boquszewski CL, Hoffman AR. Usefulness and potential pitfalls of long-acting growth hormone analogs. *Front Endocrinol (Lausanne).* 2021;12:637209. doi:10.3389/fendo.2021.637209

Patient Resources

Genetics and Rare Diseases Information Center (GARD): https://rarediseases.info.nih.gov/diseases/6552/growth-hormone-deficiency

Healthychildren.org: https://www.healthychildren.org/English/health-issues/conditions/Glands-Growth-Disorders/Pages/Growth-Hormone-Deficiency-FAQs.aspx

Hormone Health Network: https://www.hormone.org/diseases-and-conditions/growth-hormone-deficiency

Human Growth Foundation: http://www.hgfound.org

MAGIC Foundation: https://www.magicfoundation.org

Medline Plus: https://medlineplus.gov/growthdisorders.html

Merck Manual. Growth hormone deficiency in children (consumer version). https://www.merckmanuals.com/home/children-s-health-issues/hormonal-disorders-in-children/growth-hormone-deficiency-in-children

National Organization for Rare Disorders: https://rarediseases.org/rare-diseases/growth-hormone-deficiency/

Nemours Kids Health: https://kidshealth.org/en/parents/gh-deficiency.html

Pediatric Endocrine Society (Pediatric/Adolescent): https://pedsendo.org/patient-resource/growth-hormone-deficiency/

Pituitary Network Association: https://pituitary.org/knowledge-base/disorders