Growth Hormone Therapies: safety and adherence
30 May 2017 - Rio de Janeiro, Brazil
Overview

Therapy with recombinant human Growth Hormone (rhGH) is widely available and has been the most important treatment for several growth disorders in childhood since 1985. Great efforts have been made, through single centre studies and international multi-centre studies, to collect data on patient compliance and long-term safety. Both issues are still points of discussion worldwide. Achieving consensus on compliance and long-term safety is not straightforward when considering the magnitude of data required, the heterogeneity of growth disorders treated with rhGH, as well as the varying rhGH dosages and devices. Parents and caregivers involved in the care of these children still face doubts about optimal follow-up treatment. This workshop will offer the most updated scientific evidence on long-term safety of rhGH therapy and look at how to assess and improve patient compliance. Milestones and new insight on these issues will be discussed by international experts who will use the opportunity to improve how rhGH therapy is managed in childhood.

Learning objectives

By attending this live educational workshop, participants will be able to:

• Assess and improve adherence in patients on rhGH treatment
• Manage patients with growth hormone disorders (GHD) according to most updated data on long-term safety
• Manage patients with Turner Syndrome on rhGH therapy
• Assess effectiveness of rhGH therapy in SGA patients
• Arrange transition of GHD patients from childhood to adulthood

Target audience

This workshop is targeted at Latin American paediatric endocrinologists, scientists and all healthcare professionals involved in managing children with growth hormone disorders.

Chair

Paulo Ferrez Collett-Solberg
Presidente do 12º COBRAPEM - Congresso Brasileiro de Endocrinologia e Metabologia
Department of Endocrinology
University of Rio de Janeiro State (UERJ)
Rio de Janeiro-RJ, Brazil

EXCEMED designed this programme with the endorsement of the Brazilian Society of Paediatrics. (SBP - Sociedade Brasileira de Pedriatria)
CME Provider

EXCEMED is a non-profit foundation dedicated, since the last four decades, to the development of high-quality medical education programmes all over the world.

EXCEMED adheres to the guidelines and standards of the European Accreditation Council for Continuing Medical Education (EACCME®) which states that continuing medical education must be balanced, independent, objective, and scientifically rigorous.

Continuing medical education

An application has been made to the EACCME® for CME accreditation of the EXCEMED workshop “Growth Hormone Therapies: safety and adherence” to be held on 30 May 2017 in Rio de Janeiro, Brazil.

EXCEMED adheres to the principles of the Good CME Practice group (gCMEp).
Venue
This live educational workshop takes place at:
Windsor Convention & Expo Center
Rua Martinho de Mesquita, 129
Barra da Tijuca – RJ – CEP 20031-204
Rio de Janeiro, Brazil

Language
The official language of this live educational workshop is English.
Simultaneous translation from English into Portuguese and vice versa will be provided.

CME Provider
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This live educational event is allowing participants to express their own views and opinions through interactive workshops with clinical cases, panel discussions, real-time surveys with voting systems, question cards, and dedicated websites.

Access the dedicated website to:

- Download the conference application
- Enjoy easy access to:
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Any question? You can pose your question by using the question card in your folder.
Faculty

**Fernando Cassorla**  
Chief of Pediatric Endocrinology  
Institute of Maternal and Child Research  
University of Chile  
Santiago de Chile, Chile

**Pierre Chatelain**  
Professor Emeritus of Pediatrics  
College of Pediatrics  
Université Claude Bernard Lyon 1  
Lyon, France

**Paulo Ferrez Collett-Solberg**  
Presidente do 12º COBRAPEM  
Congresso Brasileiro de Endocrinologia e Metabologia  
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**Anita C.S. Hokken-Koelega**  
Professor of Paediatric Endocrinology  
Erasmus University  
Rotterdam, The Netherlands

**Alexander A.L. Jorge**  
Associate Professor of Medicine  
Endocrinology Division  
University of Sao Paulo School of Medicine  
Genetic Endocrinology Unit  
Sao Paulo, Brazil

**Lars Sävendahl**  
Professor of Pediatric Endocrinology  
Department of Woman and Child Health  
Karolinska Institutet  
Stockholm, Sweden
Tuesday, 30 May 2017

8.45 Welcome and introduction
P. Ferrez Collett-Solberg (Brazil)

Session I

Session Chair: P. Ferrez Collett-Solberg (Brazil)

9.00 L1: How to assess and improve adherence in patients on rhGH therapy
P. Ferrez Collett-Solberg (Brazil)

P. Chatelain (France)

10.00 L3: Children small for gestational age and rhGH therapy: safety and effect on cardiometabolic risk
A.C.S. Hokken-Koelega (The Netherlands)

10.30 Panel discussion

11.00 Coffee break

Session II

Session Chair: P. Ferrez Collett-Solberg (Brazil)

11.30 L4: Long-term safety of rhGH therapy in children with GH deficiency
L. Sävendahl (Sweden)

12.00 L5: Transitional period: differences of GH treatment follow-up from childhood to adulthood
A.A.L. Jorge (Brazil)

12.30 L6: Personalized approach to rhGH treatment: potential impact on outcome, adherence and safety
F. Cassorla (Chile)

13.00 Panel discussion

13.30 Lunch
14.30 Three parallel working groups: Problem solving of clinical cases

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<th>GROUP A</th>
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<td>Coordinators</td>
<td>P. Ferrez Collett-Solberg</td>
<td>A.C.S. Hokken-Koelega</td>
<td>A.A.L. Jorge</td>
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15.30 Discussion in plenary session of clinical cases from the working groups

16.30 Concluding remarks
P. Ferrez Collett-Solberg (Brazil)

16.45 End of the workshop
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The following faculty provided information regarding significant commercial relationships and/or discussions of investigational or non-EMEA/FDA approved (off-label) uses of drugs:

- **Fernando Cassorla** Declared receipt of honoraria or consultation fees from Sandoz, Debiopharm and Merck
- **Pierre Chatelain** Declared receipt of honoraria or consultation fees from Ascendis Pharma and benefit from a relationship with Merck Serono (publication)
- **Paulo Ferrez Collett-Solberg** Declared receipt of honoraria or consultation fees from Pfizer and Novo Nordisk
- **Anita C.S. Hokken-Koelega** Declared receipt of independent research grants from Pfizer, Novo Nordisk and Ipsen, of honoraria or consultation fees from Novo Nordisk Pfizer, Sandoz and Ipsen and to be member of KIGS advisory board (Pfizer post marketing registry)
- **Alexander A.L. Jorge** Declared no potential conflict of interest
- **Lars Sävendahl** Declared receipt of honoraria or consultation fees from Novo Nordisk, Merck, Pfizer and Sandoz
Fernando Cassorla received his medical degree from the University of Chile in 1973. Following his pediatric residency at the Albany Medical Center in New York, in 1976 he became a fellow in Pediatric Endocrinology at the Children’s Hospital of Philadelphia, leading to his Board Certification both in Pediatrics and in Pediatric Endocrinology in 1980. Dr Cassorla subsequently became an Associate Investigator at the Developmental Endocrinology Branch, National Institute of Child Health and Human Development in Bethesda, Maryland, rising to the position of Clinical Director of this Institute in 1990. He subsequently became Chief of Pediatric Endocrinology at the Institute of Maternal and Child Research of the University of Chile, where he has held this position since 1993. Dr Cassorla has trained over 50 fellows in this specialty, both at the NIH in USA, and at the University of Chile. He has edited 3 texts and written 36 chapters in pediatric endocrinology, has authored or co-authored 221 original articles in peer reviewed journals, and presented over 300 abstracts at scientific meetings. Dr Cassorla has received several international awards, such as the Distinguished Clinical Teacher Award and the Director´s Award at the NIH, and the Maestro Award from the Latin American Society of Pediatric Endocrinology, society which he has served both as President and Secretary General. In addition, Dr Cassorla was elected to the Chilean Academy of Medicine for a lifetime position in the year 2003.

Fernando Cassorla
Chief of Pediatric Endocrinology
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Pierre Chatelain is Professor Emeritus of Pediatrics ("Classe Exceptionnelle") at the Université Claude Bernard Lyon 1, School of Medicine of Lyon, France. He is a Paediatrician specialized in endocrinology and diabetology. From 1989 to 2014 he chaired the pediatric-endocrinology clinical unit of the university hospital in Lyon. From 2006 to 2014 he was Coordinator/Chairman of the French National Reference Centre for Rare Sex Differentiation & Development Disorders (DSD) and from 2009 to 2014 he was Chairman of the “Collège de Pédiatrie” of the Université Claude Bernard Lyon 1.


Prof. Chatelain’s main focus has been the diagnosis and treatment of abnormal fetal and postnatal growth, with contribution on Growth Hormone Releasing Hormone pioneer development as well as development of treatment with recombinant human Growth Hormone or recombinant human Insulin-Like Growth Factor 1 of specific short stature conditions. More recently he actively contributed to pioneer research on Pharmacogenomics of Growth Hormone in children.

Prof. Chatelain is author of 198 publications in International peer reviewed journals or textbooks/books in the field of endocrinology, pediatric-endocrinology, diabetology, pediatrics & science.
Paulo Ferrez Collett-Solberg received his MD degree from the Universidade Federal do Rio de Janeiro (1989). He did his residency in Pediatrics at the University of Chicago Hospitals (1994), his Fellowship in Pediatric Endocrinology at the Children’s Hospital of Philadelphia (1997) and a Doctorate in Endocrinology at the Universidade Federal do Rio de Janeiro (2006). He was Professor of Pediatrics at Duke University from 1997 to 2000. From March 2011 until March 2012 he completed a post-doctorate degree at the Laboratório de Pesquisas Clínicas e Experimentais em Biologia (BIOVASC) at Universidade do Estado do Rio de Janeiro. In March 2012 he started as an Adjunct Professor of Pediatric Endocrinology at the Universidade do Estado do Rio de Janeiro. He is a member of the Departamento of Pediatric Endocrinology from the Sociedade Brasileira de Pediatria and of the Committee of Pediatric Endocrinology from the Sociedade de Pediatria de Estado do Rio de Janeiro. He is the President of the 12th COBRAPEM.

Prof. Ferrez Collett-Solberg has experience in Pediatric Endocrinology, acting on the following subjects: adrenal, growth, short stature, and puberty disorders.
Anita C.S. Hokken-Koelega is Professor and Head of Pediatric Endocrinology Department at the Erasmus University Medical Center, Rotterdam, The Netherlands. She supervises endocrine research and large multicenter trials in the Netherlands, and is Director of the Dutch Growth Research Foundation. She is Chair of the Strategic and Finance Committee of the European Society for Paediatric Endocrinology (ESPE).

Her research focuses on endocrine and metabolic effects of acute and chronic illness in children, growth disorders and GH treatment in children with short stature due to chronic illness, born SGA, and in patients with Prader-Willi syndrome. She is also involved in the development of advanced growth-prediction models and growth charts (Growth Analyser). Professor Hokken-Koelega has published approx. 300 scientific papers in peer-reviewed journals. She has received the ASPASIA Award [The Netherlands Research Institute], the Endocrinology Award [Dutch Endocrine Society] and the European Society Pediatric Endocrinology (ESPE) Research Award 2011.
Alexander Augusto de Lima Jorge is Associate Professor of Medicine at the University of Sao Paulo (USP), School of Medicine, Brazil. He is a clinical endocrinologist as well as a translational researcher. Dr. Jorge is a principal investigator at the Developmental Endocrinology Unit and Genetic Endocrinology Unit. He is an expert in growth disorders and responsible for molecular genetic investigation of several endocrine genetic disorders at the Hospital das Clínicas de São Paulo, Brazil. His research is centered on discovering and understanding the genetic mechanisms involved in monogenic endocrine diseases, in particular those associated with growth and development disorders.
Lars Sävendahl is Professor of Pediatric Endocrinology at Karolinska Institutet, Stockholm, Sweden. He received his MD and PhD degrees from Umeå University and subspecialty training in paediatric endocrinology at the University of North Carolina (UNC) Hospitals, Chapel Hill, USA. Professor Sävendahl is a senior consultant in paediatric endocrinology at Astrid Lindgren Children’s Hospital and also the Academic Chief of the Theme for Children’s and Women’s Health at the Karolinska University Hospital in Stockholm. His major research interest is hormonal regulation of growth plate cartilage. Clinical studies related to growth are also ongoing under Professor Sävendahl’s supervision. He is the supervisor of several postgraduate research students and post-doctoral fellows working in his research group. Professor Sävendahl is active in national and international societies, served the European Society for Paediatric Endocrinology (ESPE) for many years and was the ESPE Secretary General between 2011 and 2015.
LEARNING OBJECTIVES
How to assess and improve adherence in patients on rhGH therapy

Paulo Ferrez Collett-Solberg (Brazil)

Learning objectives:
• Interpret data on adherence in patients receiving rhGH therapy
• Describe correlation between adherence and outcome of rhGH therapy
• Understand how to assess patient compliance
• Grasp how to improve patient adherence
Learning objectives:
• Clarify the impact of rhGH on adult height in Turner Syndrome
• Recognize the effects of rhGH on bone quality in Turner Syndrome
• Perceive safety of rhGH therapy in girls
• Understand long-term safety of rhGH therapy in adult women previously treated with rhGH therapy
• Interpret impact of rhGH therapy on the quality of life in Turner Syndrome
Learning objectives:

- Describe the cardiometabolic risk in SGA
- Identify the effect of rhGH on cardiometabolic risk in SGA
- Discern when to consider rhGH therapy in SGA
- Assess impact of rhGH on height in SGA
- Deduce long term safety of rhGH therapy in SGA

Children small for gestational age and rhGH therapy: safety and effect on cardiometabolic risk

Anita C.S. Hokken-Koelega (The Netherlands)
Learning objectives:

• Describe the cardiometabolic risk in SGA
• Recognize the effect of rhGH on cardiometabolic risk in SGA
• Understand when to consider rhGH therapy in SGA
• Identify the impact of rhGH on height in SGA
• Grasp long term safety of rhGH therapy in SGA
Learning objectives:

• Understand when to consider rhGH therapy during transitional period
• Describe the main targets of rhGH therapy during the transitional period
• Describe the main targets of rhGH therapy during adulthood
• Identify differences between dosage, follow-up and adherence
Learning objectives:
• Describe how to personalize rhGH therapy
• Distinguish the effect of IGF1 titration on the outcome of rhGH therapy
• Apply predictive tools for the response to rhGH therapy
• Grasp the potential impact of personalized approach on safety and adherence
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