PHARMACHILD: A European collaboration on long term outcome and pharmacovigilance for biologics used in Juvenile Idiopathic Arthritis

a PReS/ Printo project

Nico Wulffraat, Utrecht and Nicola Ruperto, Genoa

SEVENTH FRAMEWORK PROGRAMME

UMC

University Medical Center
Utrecht

EMEA expert meeting pediatric rheumatology, december 6, 2009

University Medical Center Utrecht

- ….”The need for international registry for patients with rheumatic diseases treated with biologics and methotrexate is uniformly recognised….
- Regulatory authorities should support creation of international independent registry as a source of unbiased information on long-term safety and efficiency evaluation”. (….)

Network of EU registries for biologics in JIA

University Medical Center Utrecht

Country	Abbreviation	Registry
United Kingdom	BSPAR-BNDR BSRBR	BSPAR Biologics & New Drugs Registry (JIA) and Extended Biologics Study (JIA) British Society of Rheumatology Biologics Register (RA and adults with JIA who received biologics in adulthood)
Germany	RABBIT Etanercept register JuMBO	German long-term observation of biologics in RA Register for children with JIA, receiving etanercept or MTX Register for adults with JIA who received etanercept or MTX in childhood
The Netherlands	ABC-Register	Arthritis and Biologics in Children
Finland	-	Registry of JIA patients treated with anti-TNF
Czech Republic	-	Registry of JIA patients treated with anti-TNF
Spain	-	National Etanercept registry for JIA (Valencia, Spain)
Sweden	-	Registry of JIA patients treated with anti-TNF
Italy	IPERN registry	Registry of JIA patients treated with anti-TNF
France	CEMARA	Platform for reference centers on Juvenile Arthritis
Switzerland	-	Registry of JIA patients treated with anti-TNF

We need to do better: Limitations of current registries

University Medical Center Utrecht

- *Fail to capture children who switch from one medication to another. Important data across medication changes are lost in product-specific registries.*
- *Limited number of patients with JIA. It is difficult to enroll an adequate number of JIA patients into an individual product registry for the growing number of approved products for JIA.*
- *Enrollment criteria may exclude important groups of patients.*
- *Most national registries are underpowered to detect rare severe side effects*

(from: Jeff Siegel, FDA)

Pharmacovigilance

University Medical Center Utrecht

- EMA requires pharmacovigilance and risk management of biopharmaceuticals:
  - reporting of adverse drug reactions (ADRs)
  - Proactive risk management EU risk management plan (EU-RMP) that must be submitted by the Pharma industry. In this, the (potential) risks should be described and pharmacovigilance activities proposed.
- Regulatory authorities support an international independent registry and confirm that such a registry can provide data to Pharma industry

WP1: Large scale epidemiological study

University Medical Center Utrecht

- Objective: To built up and enroll children with juvenile idiopathic arthritis (JIA) treated with biologic agents in the PRINTO/PReS web based effectiveness registry. MTX control group
- Monitoring of data.
- WEB based, PRINTO website
- Meta-analysis of >7000 JIA patients, Statistical analysis
- Selected Reported adverse events will be transferred to WP2

WP2: analysis of adverse events

University Medical Center Utrecht

- Objective: To ensure validated robust analysis of the occurrence and risk of observed serious adverse events, and in particular serious infections, malignancy, inflammatory gastrointestinal diseases, second autoimmune diseases, neurological diseases in children receiving biologics for JIA.

Progress 2010

University Medical Center Utrecht

- December 2010: first draft of protocol
- Februari 2011 submit to ethical committee
- April 2011: official start of the project
- Kick off meeting with all participants in april
- Also non EU members may contribute
- Include MTX patients as controls

Participants

University Medical Center Utrecht

Netherlands
- UMC Utrecht
- Erasmus MC Rotterdam
- NUMC Nijmegen

Italy
- Gaslini, Genoa

United Kingdom
- Uni of Manchester
- GOSH London

Germany
- Charite Berlin
- Asklepios Sankt Augustin
- WWU Münster

EMA, pediatric rheumatology section

CARRA (USA) representatives

Network of registry representatives
- Finland
- Sweden
- Denmark
- France
- Switserland
- Spain
- Hungary

UCAN and CORE initiatives