PEDIATRIC RHEUMATOLOGY

MEETING ABSTRACT Open Access

PW02-027 - CAPS and cost-effectiveness analysis project

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From 7th Congress of International Society of Systemic Auto-Inflammatory Diseases (ISSAID) Lausanne, Switerland. 22-26 May 2013

Introduction

Ultra-orphan drugs are medicines used to treat exceptionally rare diseases that are chronically debilitating or life-threatening. Low patient numbers make it difficult for pharmaceutical companies to recoup research and development costs, and consequently these medicines are generally very expensive on a per patient basis. European Union (EU) regulations promote the development of orphan drugs; but to contain costs, EU healthcare systems will increasily need the cost-effectiveness analysis (CEA) of therapies when deciding if they should be funded. Conventional methods for CEA of drugs for common conditions do not apply to ultra-orphan drugs; therefore, additional factors need to be considered.

Objectives

Using the case of ultra-orphan cryopyrin associated periodic syndromes (CAPS) currently investigated by the EuroFever registry, the RaDiCEA (Rare Diseases & Cost-Effectiveness Analysis) project is aimed at collecting prospective efficacy, safety, tolerability, treatment adherence (effectiveness data), cost of illness (COI) information, and relative effectiveness of life-long treatment strategies, and at elaborating on CEA modeling in ultra rare diseases.

Methods

Design and setting

As a EuroFever registry spin-off, a three-year, international, multicentre, observational, cost-effectiveness study will be conducted in approx. 150 CAPS patients through the Paediatric Rheumatology INternational Trials Organisation (PRINTO) network.

Participants

The EuroFever registry project (http://www.printo.it/eurofever/) involves so far 170 centres of Paediatric Rheumatology and centres of reference for all autoinflammatory diseases in 45 Countries worldwide.

Results

Main outcome measures

They will be the retention on treatment and reasons of treatment withdrawal for effectiveness. For safety, the incidence rates of anti-IL-1 agents-emergent adverse events (AEs) and serious AEs will be evaluated in comparison with incidence rates observed in CAPS subjects not exposed to anti-IL-1 agents. The bases for a cost-effectiveness model in CAPS will be set by means of a COI evaluation, and of a comparative economic evaluation of different treatment strategies in the National Health Systems'(NHS) perspectives, using CEA of direct health costs (Incremental Cost Effectiveness Ratio - ICER), and by measuring quality adjusted life years (QALY), and organ/system damage prevention up to three years.

Expected results

The RaDiCEA project will assess the long-term effectiveness of different potentially life-long treatment strategies and COI, while exploring the feasibility of a new CEA model to be generated from a rare disease (CAPS) registry.

Conclusion

As expensive medications like "biologicals" show promising results in some patients with "ultra-orphan" diseases, it becomes more and more important to have detailed information on as many patients as possible. A promising new international collaboration aims to develop a model to evaluate both costs and (long-term)

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benefits in an ultra-orphan group of diseases known as CAPS. The same model may be used in other very rare disorders.

Disclosure of interest

None declared.

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Published: 8 November 2013

doi:10.1186/1546-0096-11-S1-A168
Cite this article as: Alberighi *et al.*: PW02-027 - CAPS and cost-effectiveness analysis project. *Pediatric Rheumatology* 2013 **11**(Suppl 1): A168.