TOPP - Summary
Pulmonary hypertension (PH) reflects high blood pressure in the arteries that supply blood to the lungs, leading to shortness of breath, fatigue, and exercise intolerance. It is a rare disorder that affects both children and adults. The disease's mechanism is not fully understood, and the natural history and epidemiology of PH in children and adolescents remain poorly understood.

Introduction

- There have been recent advances in the understanding and treatment of pulmonary hypertension (PH) in adults.
- However, the natural history and epidemiology of PH in children and adolescents remain inadequately understood.

TOPP (Tracking Outcomes and Practice in Pediatric Pulmonary Hypertension) is the first international observational registry dedicated specifically to pediatric PH.

Key objectives of this prospective registry are to describe:
- Demographic and clinical characteristics of PH in children and adolescents
- Current treatment regimen
- Disease management patterns in real-world clinical practice settings

TOPP Goals

- Improve patient care in the long term
- Address lack of epidemiologic data in pediatric PH
- Provide a mechanism to support the development and implementation of diagnosis and treatment guidelines

TOPP Registry Features

- Prospective, clinician-driven, international, multi-center, observational program
- TOPP encompasses 38 specialist PH centers from 22 countries across 4 continents: TOPP encompasses 38 specialist PH centers from 22 countries across 4 continents:

- Mexico, Netherlands, Norway, Poland, Portugal, Switzerland, Turkey, UK, and USA

TOPP Detailed Objectives

- Describe the clinical and socio-demographic characteristics of pediatric PH patients at time of diagnosis and prevalent cases
- Describe co-morbidities before and at PH diagnosis
- Describe PH therapies ongoing at time of PH diagnosis and during subsequent follow-up for a minimum of three years
- Describe referral patterns before and at PH diagnosis
- Describe treatment patterns by age, gender, etiology, and other covariates
- Describe the course of disease progression and differences in outcomes according to risk factors such as associated conditions, disease severity, age at time of diagnosis and available treatment options

TOPP Registry Features

- Prospective, clinician-driven, international, multi-center, observational program
- TOPP encompasses 38 specialist PH centers across 22 countries
- TOPP registry will be carried out at 38 centers in 22 countries across 4 continents.

Patient Population

- Target inclusion is 400-450 children and adolescents with PH
- To eliminate selection bias all consecutive eligible patients will be screened and given the opportunity to participate
- Both incident (diagnosed within 3 months of enrollment) and prevalent (diagnosed more than 3 months prior to enrollment) patients will be included
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- The Executive Board may request centers to stop inclusion
- The proportion of incident to prevalent exceeds two-thirds prevalent

Outcomes

Outcomes, including survival, hospitalization, transplantation, change in therapy and interventions such as atrial septostomy and Pott's Shunt, will be described prospectively and in an observational as opposed to randomized controlled study manner.

Inclusion Criteria

- Patients aged between 3 months and 18 years at time of diagnosis
- Diagnosis confirmed by heart catheterization
- PH diagnosis must be confirmed by heart catheterization
- Patients diagnosed with PH on or after 01 January 2001
- Patients will be treated according to standard local clinical practices
- Both incident (diagnosed within 3 months of enrollment) and prevalent (diagnosed more than 3 months prior to enrollment) patients will be included
- Inclusion of patient data started in Jan 2008
- TOPP is planned to run for at least three years
- Patients will be treated according to standard local clinical practices
- Frequency of follow-up visits will be determined by the physician and the health care needs of the patient

Exclusion Criteria

- Patients with a research grant

- Diabetes mellitus
- Obstructive sleep apnea
- Obesity

- Patients with a research grant

Data Collection

An available and secure web-based Electronic Data Capture (EDC) system is used for data collection in the following categories:

- Options
- Change in therapy and interventions such as atrial septostomy and Pott's Shunt

- Patients will be included

- Both incident (diagnosed within 3 months of enrollment) and prevalent (diagnosed more than 3 months prior to enrollment) patients will be included

- Inclusion of patient data started in Jan 2008

- TOPP is planned to run for at least three years

- Patients will be treated according to standard local clinical practices

- Frequency of follow-up visits will be determined by the physician and the health care needs of the patient

- The database is available via the Internet for direct data entry by the participating centers

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