# Pediatric Formulation of bosentan in pulmonary arterial hypertension.

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No formal hypothesis is set in this study. The sample size is based on feasibility considerations.

| | |
|---|---|
| **Ethische beoordeling** | Niet van toepassing |
| **Status** | Werving nog niet gestart |
| **Type aandoening** | - |
| **Onderzoekstype** | Interventie onderzoek |

## Samenvatting

### ID

NL-OMON20918

**Bron**
NTR

**Verkorte titel**
FUTURE 4

**Aandoening**

Persistent pulmonary arterial hypertension
PPHN
bosentan
pediatrics
persisterende pulmonale arteriële hypertensie
pasgeborene

### Ondersteuning

**Primaire sponsor:** ACTELION Pharmaceuticals Ltd
Gewerbestrasse 16
CH-4123 Allschwil
Switzerland
**Overige ondersteuning:** fund = initiator = sponsor

## Onderzoeksproduct en/of interventie

## Uitkomstmaten

### Primaire uitkomstmaten

To assess the efficacy of bosentan in neonates with PPHN who are in need of continued inhaled iNO after at least 4 hours of continuous iNO treatment and to evaluate the PK, tolerability, and safety of bosentan in this patient population.

# Toelichting onderzoek

### Achtergrond van het onderzoek

To assess the efficacy of bosentan in neonates with persistent pulmonary hypertension of the newborn (PPHN) who are in need of continued inhaled nitric oxide (iNO) after at least 4 hours of continuous iNO treatment and to evaluate the pharmacokinetics (PK), tolerability, and safety of bosentan in this patient population.

### Doel van het onderzoek

No formal hypothesis is set in this study. The sample size is based on feasibility considerations.

### Onderzoeksopzet

The maximum duration of the study for an individual patient is up to 28 days from screening to end of study (EOS).

Screening period:

1. From PPHN diagnosis to randomization (maximum 7 days).

Double-blind treatment period:

1. Up to treatment failure, or;

2. Up to successful weaning from iNO, or;

3. Up to a maximum of 14 days of study drug treatment.

End of Study (EOS):

1. End of Treatment + 7 days.

Follow-up period:

1. From EOS to 60 days after last double-blind treatment administration (phone call to document any serious adverse events [SAEs]).

**Onderzoeksproduct en/of interventie**

Bosentan (2 mg/kg body weight b.i.d.) or placebo. Treatment allocation is designed to occur in a 2:1 ratio (active treatment to placebo, respectively).

Route: Nasogastric or orogastric tube.

# Contactpersonen

## Publiek

Sportlaan 8
Sofie Sledsens
Sint-Gillis-Waas 9170
Belgium
+32 473 64 10 26

## Wetenschappelijk

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# Deelname eisen

## Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

1. Signed informed consent by the parent(s) or the legal representative(s);

2. Term and near-term newborns (gestational age > 34 weeks);

3. Post natal age more or equal to 12 hours and < 7 days;

4. Weight at birth more or equal to 2,500 g;

5. Idiopathic PPHN or PPHN due to parenchymal lung disease (e.g., respiratory distress syndrome, meconium aspiration syndrome, pneumonia, sepsis without multi-organ failure);

6. Pulmonary hypertension (PH) confirmed by echocardiography:

A. Predominant extrapulmonary right-to-left or bidirectional shunting of blood at a patent foramen ovale (PFO) or patent ductus arteriosus (PDA) or;

B. Estimated right ventricular systolic pressure (RVSP) > 2/3 of systemic arterial pressure by tricuspid regurgitant jet velocity (TRJV) or by gradient across septal defect (if present) or;

C. Marked right ventricular (RV) dilation and paradoxical shift of interventricular septum.

7. Need for continued iNO at a dose > 10 ppm after at least 4h of continuous iNO treatment;

8. Last two consecutive oxygenation index (OI) values prior to randomization more or equal to 15;

9. Mechanical ventilation with fraction of inspired oxygen (FiO2) more or equal to 50%.

## Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

1. PH associated with conditions other than PPHN;

2. Immediate need for cardiac resuscitation or extracorporeal membrane oxygenation (ECMO) (profound hypoxemia [PaO2] < 30 mm Hg; OI > 40);

3. Lethal congenital anomalies;

4. Congenital diaphragmatic hernia;

5. Significant congenital heart disease or significant left to right shunt;

6. Pneumothorax;

7. Active seizures;

8. Expected duration of mechanical ventilation of less than 48 hours;

9. Mean systemic blood pressure < 35 mmHg despite therapy with volume infusions and

cardiotonic support;

10. Hepatic failure or all conditions with aspartate aminotransferase (AST) or alanine aminotransferase (ALT) values > 2 times upper limit of normal (ULN);

11. Renal function impairment such as serum creatinine > 3 times ULN or anuria;

12. Known intracranial hemorrhage grade III or IV;

13. Hemoglobin or hematocrit level < 75% of the lower limit of normal (LLN);

14. Thrombocytopenia (platelet count < 50,000 cells /microL);

15. Leukopenia (white blood cells [WBC] < 2,500 cells/ microL);

16. Any condition precluding the use of a nasogastric/orogastric tube;

17. Administration of prohibited medication prior to randomization.

# Onderzoeksopzet

## Opzet

| | |
|---|---|
| Type: | Interventie onderzoek |
| Onderzoeksmodel: | Parallel |
| Toewijzing: | Gerandomiseerd |
| Blindering: | Dubbelblind |
| Controle: | Placebo |

## Deelname

Nederland

| | |
|---|---|
| Status: | Werving nog niet gestart |
| (Verwachte) startdatum: | 01-09-2011 |
| Aantal proefpersonen: | 30 |
| Type: | Verwachte startdatum |

# Ethische beoordeling

Niet van toepassing
Soort: Niet van toepassing

# Registraties

## Opgevolgd door onderstaande (mogelijk meer actuele) registratie

Geen registraties gevonden.

## Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

## In overige registers

| Register | ID |
|---|---|
| NTR-new | NL2792 |
| NTR-old | NTR2932 |
| Ander register | Actelion Pharmaceuticals Ltd / EudraCT : AC-052-391 / 2011-000203-41; |
| ISRCTN | ISRCTN wordt niet meer aangevraagd. |

# Resultaten

**Samenvatting resultaten**
N/A