Effect, Safety and Pharmacokinetics Study of PRO051 to Treat DMD

Gepubliceerd: 07-03-2008 Laatst bijgewerkt: 13-12-2022

Ethische beoordeling	Positief advies
Status	Werving gestart
Type aandoening	-
Onderzoekstype	Interventie onderzoek

Samenvatting

ID

NL-OMON21367

Bron
NTR

Verkorte titel
N/A

Aandoening

Duchenne Muscular Dystrophy

Ondersteuning

Primaire sponsor: Prosensa
Overige ondersteuning: Sponsor

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Effect of PRO051 on dystrophin expression in muscle and on muscle strength and function

Safety and tolerability of PRO051

Pharmacokinetics of PRO051

Toelichting onderzoek

Achtergrond van het onderzoek

This is an open-label phase I/II study to assess the effect, the safety and tolerability, and the pharmacokinetics of PRO051 at different dose levels after subcutaneous administration in 12-18 patients with Duchenne muscular dystrophy.

Onderzoeksopzet

5 weeks of treatment plus 13 weeks follow up

Onderzoeksproduct en/of interventie

Subcutaneous injection of PRO051, once a week, for 5 weeks, different dose per group, 4 groups

Contactpersonen

Publiek

Prosensa
Wassenaarseweg 72

H. Akker, van den
Leiden 2333 AL
The Netherlands

Wetenschappelijk

Prosensa
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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

1. Boys aged between 5 and 16 years inclusive

2. Duchenne muscular dystrophy resulting from a mutation correctable by treatment with PRO051

3. Not ventilator dependent

4. Life expectancy of at least 6 months

5. No previous treatment with investigational medicinal treatment within 6 months prior to the study

6. Willing and able to adhere to the study visit schedule and other protocol requirements

7. Written informed consent signed (by parent(s)/legal guardian and/or the patient, according to the local regulations).

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

1. Aberrant RNA splicing and/or aberrant response to PRO051, detected by in vitro PRO051 assay during screening

2. Known presence of dystrophin in > 5% of fibres in a pre-study diagnostic muscle biopsy

3. Severe muscle abnormalities defined as increased signal intensity in >50% of the tibialis anterior muscle at MRI

4. FEV1 and/or FVC < 60% of predicted

5. Current or history of liver or renal disease

6. Acute illness within 4 weeks prior to treatment (Day 0) which may interfere with the measurements

7. Severe mental retardation which in the opinion of the investigator prohibits participation in this study

8. Severe cardiac myopathy which in the opinion of the investigator prohibits participation in this study

9. Need for mechanical ventilation

10. Creatinine concentration above 1.5 times the upper limit of normal (age corrected)

11. Serum ASAT and/or ALAT concentration(s) which suggest hepatic impairment
12. Use of anticoagulants, antithrombotics or antiplatelet agents

13. Subject has donated blood less than 90 days before the start of the study

14. Current or history of drug and/or alcohol abuse

15. Participation in another trial with an investigational product

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Anders
Toewijzing:	N.v.t. / één studie arm
Blindering:	Open / niet geblindeerd
Controle:	N.v.t. / onbekend

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	01-04-2008
Aantal proefpersonen:	18
Type:	Verwachte startdatum

Ethische beoordeling

Positief advies

Datum:	07-03-2008

Soort: Eerste indiening

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	NL1196
NTR-old	NTR1241
Ander register	EC Leuven : OG032
ISRCTN	ISRCTN wordt niet meer aangevraagd

Resultaten

Samenvatting resultaten
N/A