A Long-Term Open-Label, Safety and Superior Effectiveness Study of Cysteamine Bitartrate Delayed-release Capsules (RP103) in Patients with Cystinosis

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Comparison of steady-state cysteamine-trough WBC cystine levels between Cystagon® and RP103 over 3 months for each treatment period.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	White blood cell disorders
Study type	Interventional

Summary

ID

NL-OMON43669

Source
ToetsingOnline

Brief title
RP103-07

Condition

- White blood cell disorders

Synonym
cystinosis, Lignac-Fanconi Syndrom

Research involving
Human

Sponsors and support

Primary sponsor: Raptor Pharmaceuticals Inc.
Source(s) of monetary or material Support: Raptor Pharmaceuticals Inc.

Intervention

Keyword: - cysteamine bitartrate, - cystinosis, - delayed-release capsules

Outcome measures

Primary outcome

- To demonstrate superiority of RP103 versus Cystagon® in controlling white blood cell cystine (WBC) levels over 24 hours in patients with cystinosis.

- To assess long-term safety and tolerability of RP103.

Secondary outcome

- To compare WBC cystine measurements provided by a central laboratory versus sites* local (regional, not associated with the clinical trial) laboratories.

- To assess the long term quality of life and sleep using instruments appropriate to the subjects* age and region (US or Europe).

- To assess dosing compliance with two different cysteamine bitartrate formulations, Q6H immediate-release Cystagon® vs. Q12H delayedrelease RP103.

- To assess halitosis during Cystagon® and RP103 treatment in a subgroup of subjects.

Study description

Background summary

Cystinosis is an autosomal recessive inborn error of metabolism in which the transport of cystine out of lysosomes is reduced or absent. Cystinosis is a rare disease characterized by the accumulation of cystine and formation of crystals damaging various organs, especially the kidney, leading to renal tubular Fanconi Syndrome and progressive glomerular failure, with end stage renal failure by the end of the first decade of life.

Study objective

Comparison of steady-state cysteamine-trough WBC cystine levels between Cystagon® and RP103 over 3 months for each treatment period.

Study design

This is a long-term, open-label study of the safety, tolerability and effectiveness of Cystagon® and RP103 in pediatric and adult subjects with cystinosis. Subjects 12 years of age or older, with an average of WBC cystine level > 1 nmol * cystine/mg protein over at least 2 measurements during the previous 2 years, will be treated with their usual dose of Q6H Cystagon® for 3 months (with no dose adjustments) followed by Q12H RP103 started at a total daily dose of 70% of their Cystagon® dose for at least 4 months (with dose increase permitted only during the first month on RP103).

Intervention

Use of RP103 medication.

Study burden and risks

-nr of Blood samples: Op iedere visite wordt bloed afgenomen. Hoeveelheid is afhankelijk van de visite, tussen de 10 en 20 ml per keer.
-nr of Visits to the investigator ;Minimaal 8 bezoeken tijdens de studie, en daarna 3-maandelijkse visites met een totaal maximum van 24 maanden.
-Physical Exam: standaard lichamelijk onderzoek op iedere visite, inclusief een ECG.
-Questionnaires and diaries: Op iedere visite wordt een dagboekje bekeken. vragenlijsten mbt Kwaliteit van Leven, Vermoeidheid en een VAS score voor slikklachten.
-physical or psychological discomfort: De bloedafnames zijn wellicht vervelend. De PK/PD afnames op de vroege ochtend en/of late avond kunnen ook ongemak veroorzaken.

- Er worden geen andere bijwerkingen verwacht dan de bekende bijwerkingen die al bekend zijn bij het gebruik van Cystagon.

-De overgang van Cystagon naar RP103 zal naar verwachting een betere kwaliteit van leven geven, aangezien er door de gunstigere inname tijden een betere nachtrust verwacht wordt. Ook de reductie van het aantal pillen per inname is waarschijnlijk een verbetering voor de patient.

Contacts

Public
Raptor Pharmaceuticals Inc.

Hamilton Landing, Suite 100 7
Novato CA 94949
US
Scientific
Raptor Pharmaceuticals Inc.

Hamilton Landing, Suite 100 7
Novato CA 94949
US

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

1. Male or female with a documented diagnosis of cystinosis.

2. On a stable dose of Cystagon® for at least 21 days prior to Screening.
3. WBC cystine level > 1 nmol * cystine/mg of protein on average over at least 2 measurements
collected during the 2 years prior to Screening.
4. No clinically significant change in liver function tests, i.e. 1.5 times ULN for ALT and AST, and/or
1.5 times ULN for total bilirubin, within 6 months prior to Screening.

Exclusion criteria

1. Younger than 12 years of age.
2. Current history of the following conditions or any other health issues that make it, in the opinion
of the Investigator, unsafe for study participation:
- Inflammatory bowel disease if currently active, or prior resection of small intestine;
- Heart disease (e.g. myocardial infarction, heart failure, unstable arrhythmias, or poorly controlled
hypertension) within 90 days prior to Screening;
- Active bleeding disorder within 90 days prior to Screening;
- History of malignant disease within 2 years prior to Screening.
3. Hemoglobin level of < 9 g/dL at Screening or, in the opinion of the Investigator, a hemoglobin
level that would make it unsafe for study participation.
4. Known hypersensitivity to cysteamine and penicillamine.

Study design

Design

Study phase:	3
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	16-07-2014

Enrollment:	6
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Cystagon
Generic name:	Cysteamine Bitartrate
Registration:	Yes - NL intended use
Product type:	Medicine
Brand name:	RP103
Generic name:	Cysteamine Bitartrate

Ethics review

Approved WMO Date:	24-04-2013
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	15-08-2013
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	17-01-2014
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	21-01-2014
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	28-02-2014
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO	

Date:	28-04-2015
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	25-02-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	31-03-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	21-06-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	11-07-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
Other	NCT01733316
EudraCT	EUCTR2012-002773-64-NL

Register	ID
CCMO	NL41935.091.13