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An efficacy and safety trial of intravenous zoledronic acid in infants less than one year of age, with severe osteogenesis imperfecta

Submission date 23/09/2009	**Recruitment status** No longer recruiting	[_] Prospectively registered [_] Protocol [_] Statistical analysis plan [_] Results [_] Individual participant data [_] Record updated in last year
Registration date 04/01/2010	**Overall study status** Completed	
Last Edited 10/05/2019	**Condition category** Other	

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

ClinicalTrials.gov (NCT)
NCT00982124

Protocol serial number
SCH-INFOI

Study information

Scientific Title
An international, multicentre, open-label, efficacy and safety trial of intravenous zoledronic acid in infants less than one year of age, with severe osteogenesis imperfecta

Study objectives
This is an international, multicentre, open-label efficacy and safety trial. The primary objective is to evaluate the change in lumbar spine bone mineral density Z-score at month 24 relative to baseline using intravenous zoledronic acid compared to untreated historical controls in infants with severe osteogenensis imperfecta, who are between 2 weeks and 1 year of age, all inclusive.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Faculty of Medicine, McGill University Institutional Review Board, 08/06/2009, ref: A06-M73-06A

Study design
International multicentre open-label efficacy and safety trial

Primary study design
Interventional

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Osteogenesis imperfecta

Interventions
All patients will receive an initial infusion of zoledronic acid at 0.0125 mg per kg body weight, followed by infusions of zoledronic acid given every three months at a dose of 0.025 mg per kg body weight, administered as a 30 to 45-minute infusion. There will be a total of 10 visits over the 24 month period of time for all patients. The total number of doses is 8. All zoledronic acid patients will be hospitalised for 48 hours at the first administration of zoledronic acid to monitor for drug reactions. Ionised calcium will be measure pre-dose, 12 hours, 24, 36 and 48 hours post-dose during the hospitalisation period. Sites will call all patients at scheduled monthly visits for determination of adverse events and concomitant medications throughout the study, except for those months where there is a scheduled on-site visit. Dual energy x-ray absorptiometry (DXA) measurements of the lumbar spine and total body and radiological skeletal survey will be done at screening or at first administration of zoledronic acid, the 12 month visit (visit 6) and final visit (visit 10). Twenty infants will be enrolled; enrolment will be competitive.

Intervention Type
Drug

Phase
Phase II

Drug/device/biological/vaccine name(s)
Zoledronic acid

Primary outcome(s)
Change in lumbar spine bone mineral density Z-score at month 24 relative to baseline in zoledronic acid treated infants with severe osteogenesis imperfecta aged between 2 weeks to 1 year of age at entry, compared to historical controls. The efficacy of zoledronic acid will be demonstrated if it is shown to be a gain in Z-score of at least 1.

Key secondary outcome(s))
Effect of zoledronic acid on the change in whole body bone mineral content after 12 and 24 months of treatment relative to baseline compared to historical controls in infants 2 weeks to 1 year of age.

Completion date
31/12/2012

Eligibility

Key inclusion criteria
1. Children, male or female, 2 weeks to less than 12 months of age, at least at 38 weeks gestational age
2. Any child with phenotypic OI type II, III or IV
3. No previous treatment with bisphosphonates
4. Negative urine protein as measured by dipstick. One repeat assessment of the urine protein will be allowed. The assessment will be make 2 weeks after the first assessment and the sample must be a urine collection after a 4-hour fast

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Child

Lower age limit
2 weeks

Upper age limit
12 months

Sex
All

Key exclusion criteria
1. Blood oxygen saturation of less than 90% in room air
2. Serum creatinine level greater than 56 µmol/L
3. Any clinically significant clinical laboratory abnormalities at screening
4. Treatment with any investigational drug within the past 30 days

5. Patients who are unlikely to be able to complete the study or comply with the visit schedule
6. Any disease or planned therapy which will interfere with the procedures or data collection of this trial

Date of first enrolment
01/10/2009

Date of final enrolment
31/12/2012

Locations

Countries of recruitment
United Kingdom

Australia

Belgium

Brazil

Canada

Finland

France

South Africa

United States of America

Study participating centre
Shriners Hospitals for Children
Montreal
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Sponsor information

Organisation
Novartis Pharmaceuticals (Canada)

ROR
https://ror.org/05afs3z13

Funder(s)

Funder type
Industry

Funder Name
Novartis Pharmaceuticals Canada

Alternative Name(s)
Novartis Canada

Funding Body Type
Private sector organisation

Funding Body Subtype
For-profit companies (industry)

Location
Canada

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes