ClinicalTrials.gov Protocol Registration and Results System (PRS) Receipt
Release Date: February 16, 2024

ClinicalTrials.gov ID: NCT00418821

Study Identification

Unique Protocol ID:	OBS12874
Brief Title:	A Study of the Effect of Aldurazyme® (Laronidase) Treatment on Lactation in Female Patients With Mucopolysaccharidosis I (MPS I) and Their Breastfed Infants
Official Title:	A Multicenter, Multinational, Open-Label Study of the Effects of Aldurazyme® (Laronidase) Treatment on Lactation in Women With Mucopolysaccharidosis I (MPS I) and Their Breastfed Infants
Secondary IDs:	2007-007003-33 [EudraCT Number] ALID01803 [company study code]

Study Status

Record Verification:	February 2024
Overall Status:	Terminated [Study was conducted to fulfill a post marketing commitment (PMC). FDA acknowledged closure of PMC.]
Study Start:	October 22, 2010 [Actual]
Primary Completion:	December 21, 2022 [Actual]
Study Completion:	December 21, 2022 [Actual]

Sponsor/Collaborators

Sponsor:	Genzyme, a Sanofi Company
Responsible Party:	Sponsor
Collaborators:	BioMarin/Genzyme LLC

Oversight

U.S. FDA-regulated Drug:	
U.S. FDA-regulated Device:	
Unapproved/Uncleared Device:	No
U.S. FDA IND/IDE:	Yes
IND/IDE Information:	FDA Center: CDER IND/IDE Number: BB-IND 7334 Serial Number: 110 Has Expanded Access: No
Human Subjects Review:	Board Status:
Data Monitoring:	No
FDA Regulated Intervention:	Yes
Section 801 Clinical Trial:	Yes

Study Description

Brief Summary:	The purpose of this study is to determine if laronidase is present in the breast milk of post-partum women receiving Aldurazyme® (laronidase) and the effects of Aldurazyme (laronidase) on the growth, development, and immunologic response of their breastfed infants.
Detailed Description:	Recruitment is not limited to the facility listed; facilities not yet active may be added upon identification of a patient.

Conditions

Conditions:	Mucopolysaccharidosis I Hurler's Syndrome Hurler-Scheie Syndrome Scheie
Keywords:	

Study Design

Study Type:	Interventional
Primary Purpose:	Treatment

Study Phase:	Phase 4
Interventional Study Model:	Single Group Assignment
Number of Arms:	1
Masking:	None (Open Label)
Allocation:	N/A
Enrollment:	2 [Actual]

Arms and Interventions

Arms	Assigned Interventions
Experimental: Laronidase Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase and their infants who were breastfeed while the mothers were receiving laronidase.	Biological/Vaccine: Laronidase dose of 0.58mg/kg body weight intravenously (IV) every week Other Names: • Aldurazyme • Recombinant human alpha L iduronidase

Outcome Measures

[See Results Section.]

Eligibility

Minimum Age:	
Maximum Age:	
Sex:	Female
Gender Based:	
Accepts Healthy Volunteers:	No
Criteria:	Inclusion Criteria (Mothers):

- The patient must have a documented laronidase deficiency with a fibroblast, plasma, serum, leukocyte, or dried blood spot laronidase enzyme activity assay.
- Be pregnant, planning to breastfeed post-partum, and receiving Aldurazyme (laronidase) therapy while breastfeeding.
- Provide signed, written informed consent prior to any protocol-related procedures. Consent of a legally authorized guardian(s) is (are) required for mothers younger than 18 years of age. If a mother is under 18 years old and can understand the consent, written informed consent is required from both the mother and the authorized guardian(s).

- Provide signed, written informed consent for their infants to participate as study patients. If a mother is younger than 18 years of age, consent for mother and infant will be obtained from the legal guardian.

Exclusion Criteria (Mothers and Infants):

- Have a medical condition, serious intercurrent illness, or other extenuating circumstance that may interfere with study compliance, including all prescribed evaluations and follow-up activities.
- Have received an investigational drug within 30 days prior to study enrollment.

Contacts/Locations

Central Contact Person: Trial Transparency email recommended (Toll free number for US & Canada)
Telephone: 800-633-1610 Ext. Option 6
Email: Contact-US@sanofi.com

Central Contact Backup:

Study Officials: Clinical Sciences & Operations
Study Director
Sanofi

Locations: **Italy**
Dipartimento di Scienze Pediatriche Medico - Chirurgiche e Neuro Scienze dello Suiluppo
Rome, Italy

IPDSharing

Plan to Share IPD: Yes
Qualified researchers may request access to patient level data and related study documents including the clinical study report, study protocol with any amendments, blank case report form, statistical analysis plan, and dataset specifications. Patient level data will be anonymized and study documents will be redacted to protect the privacy of trial participants. Further details on Sanofi's data sharing criteria, eligible studies, and process for requesting access can be found at: https://vivli.org

Supporting Information:

Time Frame:
Access Criteria:
URL:

References

Citations:

Links: URL: https://doi.org/10.12891/ceog1845.2015
Description Successful pregnancy and breastfeeding in a woman with mucopolysaccharidosis type I while receiving laronidase enzyme replacement therapy

Available IPD/Information:

Documents

Study Protocol and Statistical Analysis Plan
Document Date: January 5, 2011
Uploaded: 12/01/2023 08:54

Study Results

Participant Flow

Pre-assignment Details	Study was conducted to fulfill a post marketing commitment (PMC). Upon acknowledgement of closure of PMC by FDA, the study was terminated by the Sponsor.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase and their infants who were breastfeed while the mothers were receiving laronidase.

Overall Study

	Laronidase
Started	2
Completed	1
Not Completed	1
Fulfils one of the exclusion criteria	1

Baseline Characteristics

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase and their infants who were breastfeed while the mothers were receiving laronidase.

Baseline Measures

		Laronidase
Overall Number of Participants		2
Age, Customized Measure Type: Number Unit of measure: participants	Number Analyzed	2 participants
Newborns (0-27 days)		1
Adults (between 18 and 64 years)		1
Sex: Female, Male Measure Type: Count of Participants Unit of measure: participants	Number Analyzed	2 participants
	Female	1 50%
	Male	1 50%
Race (NIH/OMB) [1] Measure Type: Count of Participants Unit of measure: participants	Number Analyzed	0 participants
	American Indian or Alaska Native	---
	Asian	---
	Native Hawaiian or Other Pacific Islander	---

		Laronidase
	Black or African American	---
	White	---
	More than one race	---
	Unknown or Not Reported	---
		[1] Measure Analysis Population Description: Based on the low enrolment numbers, no data is reported here in order to protect and maintain participant privacy/confidentiality.

Outcome Measures

1. Primary Outcome Measure:

Measure Title	Number of Lactating Women With Serum IgG Antibodies to Laronidase
Measure Description	
Time Frame	Up to 18 months

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase. Mothers received commercially available laronidase as per the country labeling information at the study site.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Lactating Women With Serum IgG Antibodies to Laronidase Measure Type: Count of Participants Unit of measure: participants	1 100%

2. Primary Outcome Measure:

Measure Title	Amount of IgG Antibody Titers to Laronidase in Lactating Women
Measure Description	
Time Frame	Baseline and Week 12

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Amount of IgG Antibody Titers to Laronidase in Lactating Women Mean (Standard Deviation) Unit of measure: antibody titer	
Baseline	25600 (NA) [1]
Week 12	51200 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

3. Primary Outcome Measure:

Measure Title	Number of Women Who Breastfed
Measure Description	
Time Frame	Up to 18 months

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Women Who Breastfed Measure Type: Count of Participants Unit of measure: participants	1 100%

4. Primary Outcome Measure:

Measure Title	Number of Women Who Were Successful at Breastfeeding
Measure Description	
Time Frame	Up to 18 months

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Women Who Were Successful at Breastfeeding Measure Type: Count of Participants Unit of measure: participants	1 100%

5. Primary Outcome Measure:

Measure Title	Number of Women Whose Breast Milk Contains Laronidase
Measure Description	
Time Frame	Up to 18 months

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Women Whose Breast Milk Contains Laronidase Measure Type: Count of Participants Unit of measure: participants	0 0%

6. Primary Outcome Measure:

Measure Title	Amount of Laronidase in the Breast Milk of Lactating Mothers With Mucopolysaccharidosis I (MPS I) Disease
Measure Description	
Time Frame	Up to 18 months

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase. There were no women whose breast milk contained laronidase hence overall number of participants analyzed is zero.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	0

No data displayed because Outcome Measure has zero total participants analyzed.

7. Primary Outcome Measure:

Measure Title	Number of Women With Abnormal Urine Glycosaminoglycans (uGAG) Levels
Measure Description	
Time Frame	Up to 18 months

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Women With Abnormal Urine Glycosaminoglycans (uGAG) Levels Measure Type: Count of Participants Unit of measure: participants	0 0%

8. Primary Outcome Measure:

Measure Title	Amount of uGAG in the Urine of Women
Measure Description	Urine samples were collected at specified intervals to measure uGAG in the urine of women. Reference range of uGAG between 2.64 - 37.65 was considered as normal. mg/g creatinine = milligram per gram of creatinine.
Time Frame	Baseline and Week 12

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Amount of uGAG in the Urine of Women Mean (Standard Deviation) Unit of measure: mg/g creatinine	
Baseline	28.13 (NA) [1]
Week 12	26.98 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

9. Primary Outcome Measure:

Measure Title	Number of Participants With Medical History of the Mother: Pre-Existing Conditions
Measure Description	
Time Frame	Baseline

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1

	Laronidase
Number of Participants With Medical History of the Mother: Pre-Existing Conditions Measure Type: Count of Participants Unit of measure: participants	
Mucopolysaccharidosis type I	1 100%
Aortic valvular insufficiency	1 100%
Stipsi	1 100%
Carpal tunnel syndrome	1 100%

10. Primary Outcome Measure:

Measure Title	Physical Examination Findings of the Mother
Measure Description	Physical examination was performed at specified intervals. Physical examination included the following physical observations: general appearance, skin, head, ears, eyes, nose, and throat, lymph nodes, abdomen, extremities/joints, neurological, mental status, and the following, if appropriate, breasts, external genitalia, pelvic, and rectal.
Time Frame	Up to 18 months

Analysis Population Description

Based on the low enrolment numbers, no data is reported here in order to protect and maintain participant privacy/confidentiality.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	0

No data displayed because Outcome Measure has zero total participants analyzed.

11. Primary Outcome Measure:

Measure Title	Temperature of the Mother
Measure Description	

Time Frame	Baseline and Week 12

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase. Temperature was not measured at Week 12.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

		Laronidase
Overall Number of Participants Analyzed		1
Temperature of the Mother Mean (Standard Deviation) Unit of measure: Celsius	[Not specified]	
Baseline	Number Analyzed	1 participants
		35.8 (NA) [1]
Week 12	Number Analyzed	0 participants
		---

[1] Standard deviation cannot be derived for a single participant.

12. Primary Outcome Measure:

Measure Title	Heart Rate of the Mother
Measure Description	
Time Frame	Baseline and Week 12

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase. Heart rate was not measured at Week 12.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

		Laronidase
Overall Number of Participants Analyzed		1
Heart Rate of the Mother Mean (Standard Deviation) Unit of measure: beats per minute	[Not specified]	
Baseline	Number Analyzed	1 participants
		90 (NA) [1]
Week 12	Number Analyzed	0 participants
		---

[1] Standard deviation cannot be derived for a single participant.

13. Primary Outcome Measure:

Measure Title	Respiratory Rate of the Mother
Measure Description	
Time Frame	Baseline and Week 12

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase. Respiratory rate was not measured at Baseline and Week 12.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	0

No data displayed because Outcome Measure has zero total participants analyzed.

14. Primary Outcome Measure:

Measure Title	Blood Pressure of the Mother
Measure Description	Systolic blood pressure (SBP) and diastolic blood pressure (DBP) were measured at specified timepoints.
Time Frame	Baseline and Week 12

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase. Blood pressure was not measured at Week 12.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

		Laronidase
Overall Number of Participants Analyzed		1
Blood Pressure of the Mother Mean (Standard Deviation) Unit of measure: millimeters of mercury	[Not specified]	
SBP at Baseline	Number Analyzed	1 participants
		125 (NA) [1]
DBP at Baseline	Number Analyzed	1 participants
		80 (NA) [1]
SBP at Week 12	Number Analyzed	0 participants

DBP at Week 12	Number Analyzed	0 participants
		---

[1] Standard deviation cannot be derived for a single participant.

15. Primary Outcome Measure:

Measure Title	Weight of the Mother

Measure Description	
Time Frame	Baseline and Week 12

Analysis Population Description

Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Weight of the Mother Mean (Standard Deviation) Unit of measure: kilograms	
Baseline	58.5 (NA) [1]
Week 12	51 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

16. Primary Outcome Measure:

Measure Title	Height of the Mother
Measure Description	
Time Frame	Baseline

Analysis Population Description

Based on the low enrolment numbers, no data is reported here in order to protect and maintain participant privacy/confidentiality.

Reporting Groups

	Description
Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	0

No data displayed because Outcome Measure has zero total participants analyzed.

17. Primary Outcome Measure:

Measure Title	Number of Infants With Abnormal uGAG Levels
Measure Description	
Time Frame	Up to 72 weeks

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Infants With Abnormal uGAG Levels Measure Type: Count of Participants Unit of measure: participants	1 100%

18. Primary Outcome Measure:

Measure Title	Amount of uGAG in the Urine of Infants
Measure Description	Urine samples were collected at specified intervals to measure uGAG in the urine of infant. Reference range of uGAG between 30 - 300 was considered as normal.
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, Week 60, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase. No sample was collected at Week 60.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

		Laronidase
Overall Number of Participants Analyzed		1
Amount of uGAG in the Urine of Infants Mean (Standard Deviation) Unit of measure: mg/g creatinine	[Not specified]	
Baseline	Number Analyzed	1 participants
		76 (NA) [1]
Week 12	Number Analyzed	1 participants
		194 (NA) [1]
Week 24	Number Analyzed	1 participants
		29 (NA) [1]
Week 36	Number Analyzed	1 participants
		55 (NA) [1]
Week 48	Number Analyzed	1 participants
		45 (NA) [1]
Week 60	Number Analyzed	0 participants

Week 72	Number Analyzed	1 participants
		44 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

19. Primary Outcome Measure:

Measure Title	Number of Participants With Medical History of the Infant:Pre-Existing Conditions
Measure Description	
Time Frame	Baseline

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Participants With Medical History of the Infant:Pre-Existing Conditions Measure Type: Count of Participants Unit of measure: participants	
Extremities/Joints (clinodactily hands)	1 100%
Skin (bilateral transverse palmar crease)	1 100%

20. Primary Outcome Measure:

Measure Title	Number of Infants With Abnormal Physical Finding
Measure Description	Physical examination included the following physical observations: general appearance, skin, lymph nodes, heart, lungs, abdomen, extremities/joints, neurological, mental status, breasts, external genitalia, pelvic, rectal, and Heent.
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Infants With Abnormal Physical Finding Measure Type: Number Unit of measure: participants	
Extremities/Joints at Baseline (clinodactily hands)	1
Skin at Baseline (bilateral transverse palmar crease)	1
Skin at Week 12 (bilateral transverse palmar crease)	1
Skin at Week 24 (bilateral transverse palmar crease)	1
Skin at Week 36 (bilateral transverse palmar crease almost disappeared)	1
Lungs at Week 48 (grunting)	1
Skin at Week 72 (bilateral transverse palmar crease)	1

21. Primary Outcome Measure:

Measure Title	Heart Rate of the Infant
Measure Description	Heart rate was measured at specified timepoints.
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase. Heart rate was not measured at Baseline and Week 24.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1

		Laronidase
Heart Rate of the Infant Mean (Standard Deviation) Unit of measure: beats per minute	[Not specified]	
Baseline	Number Analyzed	0 participants

Week 12	Number Analyzed	1 participants
		121 (NA) [1]
Week 24	Number Analyzed	0 participants

Week 36	Number Analyzed	1 participants
		102 (NA) [1]
Week 48	Number Analyzed	1 participants
		120 (NA) [1]
Week 72	Number Analyzed	1 participants
		119 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

22. Primary Outcome Measure:

Measure Title	Respiratory Rate of the Infant
Measure Description	Respiratory rate was measured at specified timepoints.
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase. Respiratory rate was not measured at Baseline and Week 24.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

		Laronidase
Overall Number of Participants Analyzed		1
Respiratory Rate of the Infant Mean (Standard Deviation) Unit of measure: breaths/min	[Not specified]	
Baseline	Number Analyzed	0 participants

Week 12	Number Analyzed	1 participants
		18 (NA) [1]
Week 24	Number Analyzed	0 participants

Week 36	Number Analyzed	1 participants
		13 (NA) [1]
Week 48	Number Analyzed	1 participants
		16 (NA) [1]
Week 72	Number Analyzed	1 participants
		23 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

23. Primary Outcome Measure:

Measure Title	Blood Pressure of the Infant
Measure Description	Systolic blood pressure (SBP) and diastolic blood pressure (DBP) were measured at specified timepoints.
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase. Blood pressure was not measured at Baseline, Week 12, and Week 24.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

		Laronidase
Overall Number of Participants Analyzed		1
Blood Pressure of the Infant Mean (Standard Deviation) Unit of measure: millimeters of mercury	[Not specified]	
Baseline SBP	Number Analyzed	0 participants

Week 12 SBP	Number Analyzed	0 participants

Week 24 SBP	Number Analyzed	0 participants

Week 36 SBP	Number Analyzed	1 participants
		90 (NA) [1]
Week 48 SBP	Number Analyzed	1 participants
		87 (NA) [1]
Week 72 SBP	Number Analyzed	1 participants
		90 (NA) [1]
Baseline DBP	Number Analyzed	0 participants

Week 12 DBP	Number Analyzed	0 participants

Week 24 DBP	Number Analyzed	0 participants

		Laronidase
Week 36 DBP	Number Analyzed	1 participants
		48 (NA) [1]
Week 48 DBP	Number Analyzed	1 participants
		52 (NA) [1]
Week 72 DBP	Number Analyzed	1 participants
		54 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

24. Primary Outcome Measure:

Measure Title	Weight of the Infant
Measure Description	
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Weight of the Infant Mean (Standard Deviation) Unit of measure: kilograms	
Baseline	2.5 (NA) [1]
Week 12	5.8 (NA) [1]
Week 24	7.8 (NA) [1]

	Laronidase
Week 36	9.2 (NA) [1]
Week 48	9.9 (NA) [1]
Week 72	10.8 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

25. Primary Outcome Measure:

Measure Title	Height of the Infant
Measure Description	
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Height of the Infant Mean (Standard Deviation) Unit of measure: centimeter	
Baseline	50.0 (NA) [1]
Week 12	64.0 (NA) [1]
Week 24	66.5 (NA) [1]
Week 36	72.0 (NA) [1]
Week 48	76.5 (NA) [1]

	Laronidase
Week 72	78.0 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

26. Primary Outcome Measure:

Measure Title	Temperature of the Infant
Measure Description	
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase. Temperature was not measured at Baseline and Week 12.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

		Laronidase
Overall Number of Participants Analyzed		1
Temperature of the Infant Mean (Standard Deviation) Unit of measure: Celcius	[Not specified]	
Baseline	Number Analyzed	0 participants

Week 12	Number Analyzed	0 participants

Week 24	Number Analyzed	1 participants
		36.5 (NA) [1]
Week 36	Number Analyzed	1 participants
		36.2 (NA) [1]

		Laronidase
Week 48	Number Analyzed	1 participants
		35.9 (NA) [1]
Week 72	Number Analyzed	1 participants
		36.3 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

27. Primary Outcome Measure:

Measure Title	Head Circumference of the Infant
Measure Description	
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Head Circumference of the Infant Mean (Standard Deviation) Unit of measure: centimeter	
Baseline	33 (NA) [1]
Week 12	39 (NA) [1]
Week 24	42 (NA) [1]
Week 36	43.2 (NA) [1]

	Laronidase
Week 48	47 (NA) [1]
Week 72	45 (NA) [1]

[1] Standard deviation cannot be derived for a single participant.

28. Primary Outcome Measure:

Measure Title	Number of Participants With Normal Overall Assessment Measured Using Denver II Developmental Screening Scores
Measure Description	Infant development was assessed with the Denver II Developmental Screening Test. It consisted of 5 areas/subscore i.e., test behavior, personal-social, fine motor, language, and gross motor. The number of participants with normal overall assessment measured using Denver II Developmental Screening Scores have been reported by visit.
Time Frame	Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Participants With Normal Overall Assessment Measured Using Denver II Developmental Screening Scores Measure Type: Count of Participants Unit of measure: participants	
Week 12	1 100%
Week 24	1 100%
Week 36	1 100%
Week 48	1 100%
Week 72	1 100%

29. Primary Outcome Measure:

Measure Title	Number of Infants With IgM and IgG Antibodies to Laronidase Present at Any Time Point
Measure Description	
Time Frame	Baseline, Week 12, Week 24, Week 36, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Infants With IgM and IgG Antibodies to Laronidase Present at Any Time Point Measure Type: Count of Participants Unit of measure: participants	
IgG at Baseline	1 100%
IgG at Week 12	0 0%
IgG at Week 24	0 0%
IgG at Week 36	0 0%
IgG at Week 72	0 0%
IgM at Baseline	0 0%
IgM at Week 12	0 0%
IgM at Week 24	0 0%
IgM at Week 36	0 0%
IgM at Week 72	0 0%

30. Primary Outcome Measure:

Measure Title	Time to Development of IgM and IgG Antibodies to Laronidase
Measure Description	
Time Frame	up to Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase. No IgM and IgG antibody was detected during the study, hence time is not-evaluable.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	0

No data displayed because Outcome Measure has zero total participants analyzed.

31. Primary Outcome Measure:

Measure Title	Amount of IgG and IgM Antibody Titers to Laronidase
Measure Description	
Time Frame	Baseline, Week 12, Week 24, Week 36, and Week 72

Analysis Population Description

Infants who were breastfeed while the mothers were receiving laronidase. No IgG antibody was detected at Week 12, Week 24, Week 36, and Week 72. No IgM was detected at Baseline, Week 12, Week 24, Week 36, and Week 72.

Reporting Groups

	Description
Laronidase	Infants who were breastfeed while the mothers were receiving laronidase.

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1

		Laronidase
Amount of IgG and IgM Antibody Titers to Laronidase Mean (Standard Deviation) Unit of measure: antibody titer	[Not specified]	
IgG at Baseline	Number Analyzed	1 participants
		6400 (NA) [1]
IgG at Week 12	Number Analyzed	0 participants

IgG at Week 24	Number Analyzed	0 participants

IgG at Week 36	Number Analyzed	0 participants

IgG at Week 72	Number Analyzed	0 participants

IgM at Baseline	Number Analyzed	0 participants

IgM at Week 12	Number Analyzed	0 participants

IgM at Week 24	Number Analyzed	0 participants

IgM at Week 36	Number Analyzed	0 participants

IgM at Week 72	Number Analyzed	0 participants
		---

[1] Standard deviation cannot be derived for a single participant.

Reported Adverse Events

Time Frame	Up to approximately 18 months for mother and 72 weeks for the infant.
Adverse Event Reporting Description	[Not specified]

Reporting Groups

	Description
Mothers Treated With Laronidase	Mothers treated with laronidase who intended to breastfeed their infants while receiving laronidase.
Infants Who Were Breastfeed	Infants who were breastfeed while the mothers were receiving laronidase.

All-Cause Mortality

	Mothers Treated With Laronidase	Infants Who Were Breastfeed
	Affected/At Risk (%)	Affected/At Risk (%)
Total All-Cause Mortality	0/1 (0%)	0/1 (0%)

Serious Adverse Events

	Mothers Treated With Laronidase	Infants Who Were Breastfeed
	Affected/At Risk (%)	Affected/At Risk (%)
Total	0/1 (0%)	0/1 (0%)

Other Adverse Events

Frequency Threshold Above Which Other Adverse Events are Reported: 0%

	Mothers Treated With Laronidase	Infants Who Were Breastfeed
	Affected/At Risk (%)	Affected/At Risk (%)
Total	1/1 (100%)	1/1 (100%)
Cardiac disorders		
Tachycardia [A] †	1/1 (100%)	0/1 (0%)
Infections and infestations		
Influenza [A] †	1/1 (100%)	0/1 (0%)
Rhinitis [A] †	0/1 (0%)	1/1 (100%)
Psychiatric disorders		

	Mothers Treated With Laronidase	Infants Who Were Breastfeed
	Affected/At Risk (%)	Affected/At Risk (%)
Perinatal depression [A] †	1/1 (100%)	0/1 (0%)
Skin and subcutaneous tissue disorders		
Dermatitis atopic [A] †	0/1 (0%)	1/1 (100%)

† Indicates events were collected by systematic assessment.
A Term from vocabulary, MedDRA

Limitations and Caveats

Study was conducted to fulfill a post marketing commitment (PMC). Upon acknowledgement of closure of PMC by FDA, the study was terminated by the Sponsor.

More Information

Certain Agreements:

Principal Investigators are NOT employed by the organization sponsoring the study.

There IS an agreement between the Principal Investigator and the Sponsor (or its agents) that restricts the PI's rights to discuss or publish trial results after the trial is completed.

Results Point of Contact:

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