

ClinicalTrials.gov PRS **DRAFT Receipt (Working Version)**

Last Update: 12/11/2023 09:34

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

 NOTE : Brief Title should have no more than 120 characters.

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: December 2023

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 No  
Device:

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: <ul style="list-style-type: none"><li>• Aldurazyme</li><li>• Recombinant human alpha L iduronidase</li></ul>

## 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 Sviluppo  
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:

🔗 NOTE : IPD Sharing Time Frame has not been entered.

🔗 NOTE : IPD Sharing Access Criteria has not been entered.

## 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 breastfed 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 breastfed while the mothers were receiving laronidase.

### Baseline Measures

◆ NOTE : A Study Specific Baseline Measure for an Outcome Measure has not been entered.

		Laronidase
Overall Number of Participants		2
<b>Age, Customized</b> Measure Number Type: participants Unit of measure: participants	Number Analyzed	2 participants
Newborns (0-27 days)		1
Adults (between 18 and 64 years)		1
<b>Sex: Female, Male</b> Measure Count of Type: Participants Unit of measure: participants	Number Analyzed	2 participants
	Female	1 50%
	Male	1 50%
<b>Race (NIH/OMB) [1]</b> Measure Count of Type: 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	 NOTE : An Outcome Measure Description has not been entered.
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	 NOTE : An Outcome Measure Description has not been entered.
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) <sup>[1]</sup>
Week 12	51200 (NA) <sup>[1]</sup>

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

## 3. Primary Outcome Measure:

Measure Title	Number of Women Who Breastfed
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
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	 NOTE : An Outcome Measure Description has not been entered.
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	 NOTE : An Outcome Measure Description has not been entered.
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	 NOTE : An Outcome Measure Description has not been entered.
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
Amount of Laronidase in the Breast Milk of Lactating Mothers With Mucopolysaccharidosis I (MPS I) Disease Mean (Standard Deviation) Unit of measure: milligrams	NA (NA) <sup>[1]</sup>

[1] Not applicable as there were no women whose breast milk contained laronidase.

### 7. Primary Outcome Measure:

Measure Title	Number of Women With Abnormal Urine Glycosaminoglycans (uGAG) Levels
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
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) <sup>[1]</sup>
Week 12	26.98 (NA) <sup>[1]</sup>

[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
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
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
Number of Participants With Medical History of the Mother 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	 NOTE : An Outcome Measure Description has not been entered.
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
Temperature of the Mother Mean (Standard Deviation) Unit of measure: Celsius	
Baseline	35.8 (NA) <sup>[1]</sup>
Week 12	NA (NA) <sup>[2]</sup>

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

[2] Temperature was not measured.

**12. Primary Outcome Measure:**

Measure Title	Heart Rate of the Mother
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
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
Heart Rate of the Mother Mean (Standard Deviation) Unit of measure: beats per minute	
Baseline	90 (NA) <sup>[1]</sup>
Week 12	NA (NA) <sup>[2]</sup>

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

[2] Heart rate was not measured.

### 13. Primary Outcome Measure:

Measure Title	Respiratory Rate of the Mother
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
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

	Laronidase
Respiratory Rate of the Mother Mean (Standard Deviation) Unit of measure: breaths/min	
Baseline	NA (NA) <sup>[1]</sup>
Week 12	NA (NA) <sup>[1]</sup>

[1] Respiratory rate was not measured.

#### 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.

#### 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	
SBP at Baseline	125 (NA) <sup>[1]</sup>
DBP at Baseline	80 (NA) <sup>[1]</sup>
SBP at Week 12	NA (NA) <sup>[2]</sup>

	Laronidase
DBP at Week 12	NA (NA) <sup>[2]</sup>

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

[2] Blood pressure was not measured.

#### 15. Primary Outcome Measure:

Measure Title	Weight of the Mother
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
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) <sup>[1]</sup>
Week 12	51 (NA) <sup>[1]</sup>

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

#### 16. Primary Outcome Measure:

Measure Title	Height of the Mother
Measure Description	 NOTE : An Outcome Measure Description has not been entered.

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	 NOTE : An Outcome Measure Description has not been entered.
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 breastfed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfed 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	
Baseline	76 (NA) <sup>[1]</sup>
Week 12	194 (NA) <sup>[1]</sup>
Week 24	29 (NA) <sup>[1]</sup>
Week 36	55 (NA) <sup>[1]</sup>
Week 48	45 (NA) <sup>[1]</sup>
Week 60	NA (NA) <sup>[2]</sup>
Week 72	44 (NA) <sup>[1]</sup>

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

[2] No sample was collected.

**19. Primary Outcome Measure:**

Measure Title	Number of Participants With Medical History of the Infant
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
Time Frame	Baseline

Analysis Population Description

Infants who were breastfed while the mothers were receiving laronidase.

Reporting Groups

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

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Number of Participants With Medical History of the Infant 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 breastfed while the mothers were receiving laronidase.

Reporting Groups

	Description
Laronidase	Infants who were breastfed 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.

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	
Baseline	NA (NA) <sup>[1]</sup>
Week 12	121 (NA) <sup>[2]</sup>
Week 24	NA (NA) <sup>[1]</sup>
Week 36	102 (NA) <sup>[2]</sup>
Week 48	120 (NA) <sup>[2]</sup>
Week 72	119 (NA) <sup>[2]</sup>

[1] Heart rate was not measured.

[2] 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 breastfed while the mothers were receiving laronidase.

### Reporting Groups

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

### Measured Values

	Laronidase
Overall Number of Participants Analyzed	1

	Laronidase
Respiratory Rate of the Infant Mean (Standard Deviation) Unit of measure: breaths/min	
Baseline	NA (NA) <sup>[1]</sup>
Week 12	18 (NA) <sup>[2]</sup>
Week 24	NA (NA) <sup>[1]</sup>
Week 36	13 (NA) <sup>[2]</sup>
Week 48	16 (NA) <sup>[2]</sup>
Week 72	23 (NA) <sup>[2]</sup>

[1] Respiratory rate was not measured.

[2] 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 breastfed while the mothers were receiving laronidase.

#### Reporting Groups

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

#### Measured Values

	Laronidase
Overall Number of Participants Analyzed	1

	Laronidase
<b>Blood Pressure of the Infant</b> Mean (Standard Deviation) Unit of measure: millimeters of mercury	
Baseline SBP	NA (NA) <sup>[1]</sup>
Week 12 SBP	NA (NA) <sup>[1]</sup>
Week 24 SBP	NA (NA) <sup>[1]</sup>
Week 36 SBP	90 (NA) <sup>[2]</sup>
Week 48 SBP	87 (NA) <sup>[2]</sup>
Week 72 SBP	90 (NA) <sup>[2]</sup>
Baseline DBP	NA (NA) <sup>[1]</sup>
Week 12 DBP	NA (NA) <sup>[1]</sup>
Week 24 DBP	NA (NA) <sup>[1]</sup>
Week 36 DBP	48 (NA) <sup>[2]</sup>
Week 48 DBP	52 (NA) <sup>[2]</sup>
Week 72 DBP	54 (NA) <sup>[2]</sup>

[1] Blood pressure was not measured.

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

#### 24. Primary Outcome Measure:

Measure Title	Weight of the Infant
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

#### Analysis Population Description

Infants who were breastfed while the mothers were receiving laronidase.

### Reporting Groups

	Description
Laronidase	Infants who were breastfed 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) <sup>[1]</sup>
Week 12	5.8 (NA) <sup>[1]</sup>
Week 24	7.8 (NA) <sup>[1]</sup>
Week 36	9.2 (NA) <sup>[1]</sup>
Week 48	9.9 (NA) <sup>[1]</sup>
Week 72	10.8 (NA) <sup>[1]</sup>

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

### 25. Primary Outcome Measure:

Measure Title	Height of the Infant
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

### Analysis Population Description

Infants who were breastfed while the mothers were receiving laronidase.

### Reporting Groups

	Description
Laronidase	Infants who were breastfed 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) <sup>[1]</sup>
Week 12	64.0 (NA) <sup>[1]</sup>
Week 24	66.5 (NA) <sup>[1]</sup>
Week 36	72.0 (NA) <sup>[1]</sup>
Week 48	76.5 (NA) <sup>[1]</sup>
Week 72	78.0 (NA) <sup>[1]</sup>

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

**26. Primary Outcome Measure:**

Measure Title	Temperature of the Infant
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
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

	Laronidase
Temperature of the Infant Mean (Standard Deviation) Unit of measure: Celcius	
Baseline	NA (NA) <sup>[1]</sup>
Week 12	NA (NA) <sup>[1]</sup>
Week 24	36.5 (NA) <sup>[2]</sup>
Week 36	36.2 (NA) <sup>[2]</sup>
Week 48	35.9 (NA) <sup>[2]</sup>
Week 72	36.3 (NA) <sup>[2]</sup>

[1] Temperature was not measured.

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

**27. Primary Outcome Measure:**

Measure Title	Head Circumference of the Infant
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
Time Frame	Baseline, Week 12, Week 24, Week 36, Week 48, and Week 72

Analysis Population Description

Infants who were breastfed while the mothers were receiving laronidase.

Reporting Groups

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

Measured Values

	Laronidase
Overall Number of Participants Analyzed	1

	Laronidase
Head Circumference of the Infant Mean (Standard Deviation) Unit of measure: centimeter	
Baseline	33 (NA) <sup>[1]</sup>
Week 12	39 (NA) <sup>[1]</sup>
Week 24	42 (NA) <sup>[1]</sup>
Week 36	43.2 (NA) <sup>[1]</sup>
Week 48	47 (NA) <sup>[1]</sup>
Week 72	45 (NA) <sup>[1]</sup>

[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

	Laronidase
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	 NOTE : An Outcome Measure Description has not been entered.
Time Frame	Baseline, Week 12, Week 24, Week 36, and Week 72

#### Analysis Population Description

Infants who were breastfed while the mothers were receiving laronidase.

#### Reporting Groups

	Description
Laronidase	Infants who were breastfed 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%

	Laronidase
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	 NOTE : An Outcome Measure Description has not been entered.
Time Frame	up to Week 72

#### Analysis Population Description

Infants who were breastfed while the mothers were receiving laronidase.

#### Reporting Groups

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

#### Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Time to Development of IgM and IgG Antibodies to Laronidase Mean (Standard Deviation) Unit of measure: weeks	NA (NA) <sup>[1]</sup>

[1] No IgM and IgG antibody was detected during the study, hence time is not-evaluable.

### 31. Primary Outcome Measure:

Measure Title	Amount of IgG and IgM Antibody Titers to Laronidase
Measure Description	 NOTE : An Outcome Measure Description has not been entered.
Time Frame	Baseline, Week 12, Week 24, Week 36, and Week 72

#### Analysis Population Description

Infants who were breastfed while the mothers were receiving laronidase.

#### Reporting Groups

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

#### Measured Values

	Laronidase
Overall Number of Participants Analyzed	1
Amount of IgG and IgM Antibody Titers to Laronidase Mean (Standard Deviation) Unit of measure: antibody titer	
IgG at Baseline	6400 (NA) <sup>[1]</sup>
IgG at Week 12	NA (NA) <sup>[2]</sup>
IgG at Week 24	NA (NA) <sup>[2]</sup>
IgG at Week 36	NA (NA) <sup>[2]</sup>
IgG at Week 72	NA (NA) <sup>[2]</sup>
IgM at Baseline	NA (NA) <sup>[3]</sup>
IgM at Week 12	NA (NA) <sup>[3]</sup>
IgM at Week 24	NA (NA) <sup>[3]</sup>
IgM at Week 36	NA (NA) <sup>[3]</sup>
IgM at Week 72	NA (NA) <sup>[3]</sup>

- [1] Standard deviation cannot be derived for a single participant.
- [2] No IgG antibody detected.
- [3] No IgM antibody detected.

## 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 <sup>A</sup> †	1/1 (100%)	0/1 (0%)

	Mothers Treated With Laronidase	Infants Who Were Breastfeed
	Affected/At Risk (%)	Affected/At Risk (%)
Infections and infestations		
Influenza <sup>A</sup> †	1/1 (100%)	0/1 (0%)
Rhinitis <sup>A</sup> †	0/1 (0%)	1/1 (100%)
Psychiatric disorders		
Perinatal depression <sup>A</sup> †	1/1 (100%)	0/1 (0%)
Skin and subcutaneous tissue disorders		
Dermatitis atopic <sup>A</sup> †	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:

Name/Official Title: Trial Transparency Team

Organization: Sanofi aventis recherche & développement

Phone: 800-633-1610 Ext: 6#

Email: Contact-US@sanofi.com