ClinicalTrials.gov Protocol and Results Registration System (PRS) Receipt
Release Date: 08/22/2014

ClinicalTrials.gov ID: NCT00891202

Study Identification

Unique Protocol ID: GZGD02507

Brief Title: A Study of Eliglustat Tartrate (Genz-112638) in Patients With Gaucher Disease (ENGAGE)

Official Title: A Phase 3, Randomized, Double-Blind, Placebo-Controlled, Multi-Center Study Confirming the Efficacy and Safety of Genz-112638 in Patients With Gaucher Disease Type 1 (ENGAGE)

Secondary IDs: 2008-005222-37 [EudraCT Number]
EFC12813 [Sanofi]

Study Status

Record Verification: August 2014

Overall Status: Active, not recruiting

Study Start: November 2009

Primary Completion: July 2012 [Actual]

Study Completion: November 2015 [Anticipated]

Sponsor/Collaborators

Sponsor: Genzyme, a Sanofi Company

Responsible Party: Sponsor

Collaborators:

Oversight

FDA Regulated?: Yes

IND/IDE Protocol?:	Yes
IND/IDE Information:	Grantor: CDER IND/IDE Number: 67,589 Serial Number: 0064 Has Expanded Access? No
Review Board:	Approval Status: Approved Board Name: Board Affiliation: Phone: Email:
Data Monitoring?:	Yes
Oversight Authorities:	United States: Food and Drug Administration India: Drugs Controller General of India Russia: Ministry of Health of the Russian Federation Netherlands: Medicines Evaluation Board (MEB) Canada: Health Canada United Kingdom: Medicines and Healthcare Products Regulatory Agency Mexico: Ministry of Health Bulgaria: Bulgarian Drug Agency Israel: Israeli Health Ministry Pharmaceutical Administration Jordan: Ethical Committee Tunisia: Office of Pharmacies and Medicines Colombia: INVIMA Instituto Nacional de Vigilancia de Medicamentos y Alimentos Lebanon: Ministry of Public Health Chile: Ministry of Health Serbia and Montenegro: Agency for Drugs and Medicinal Devices

Study Description

Brief Summary:	This Phase 3 study is designed to confirm the efficacy and safety of eliglustat tartrate (Genz-112638) in participants with Gaucher disease Type 1.
Detailed Description:	Gaucher disease is characterized by lysosomal accumulation of glucosylceramide due to impaired glucosylceramide hydrolysis. Type 1 Gaucher disease, the most common form accounts for greater than (>) 90% of cases and does not involve the central nervous system (CNS). Typical manifestations of Type 1 Gaucher disease include splenomegaly, hepatomegaly, thrombocytopenia, anemia, skeletal pathology and decreased quality of life. The disease manifestations are caused by the accumulations of glucosylceramide (storage material) in Gaucher cells which have infiltrated the spleen and liver as well as other tissue. Eliglustat tartrate is a small molecule developed as an oral therapy which acts to specifically inhibit production of this storage material in Gaucher cells.

This study is designed to determine the efficacy, safety, and pharmacokinetics (PK) of eliglustat tartrate in adult participants (>16 years) with Gaucher disease Type 1. The study consists of 2 periods: The Double-Blind Primary Analysis Period (Day 1 to Week 39) and the Open-Label Period (post-Week 39 [Day 1 of the Open-Label Period] through study completion).

Conditions

Conditions: Gaucher Disease, Type 1

Keywords: Gaucher,
beta-glucosidase,
acid ß-glucosidase,
glucocerebrosidase,
glucosylceramide,
D-glucosyl-N-acylsphingosine glucohydrolase,
substrate reduction therapy

Study Design

Study Type: Interventional

Primary Purpose: Treatment

Study Phase: Phase 3

Intervention Model: Parallel Assignment

Number of Arms: 2

Masking: Double Blind (Subject, Caregiver, Investigator, Outcomes Assessor)

Allocation: Randomized

Endpoint Classification: Safety/Efficacy Study

Enrollment: 40 [Actual]

Arms and Interventions

Arms	Assigned Interventions
Experimental: Active Eliglustat tartrate	Drug: Eliglustat tartrate Eliglustat tartrate capsule as a single 50 milligram (mg) dose on Day 1 followed by eliglustat tartrate 50 mg capsule twice daily (BID) from Day 2 to Week 4, and then either eliglustat tartrate 50 mg capsule BID (in participants who had a Genz-99067 [active moiety of eliglustat tartrate in plasma] trough plasma concentration greater than or equal to [>=]

Arms	Assigned Interventions
	5 nanogram per milliliter [ng/mL]) or eliglustat tartrate 100 mg capsule BID (in participants who had a Genz-99067 trough plasma concentration less than [<] 5 ng/mL), up to Week 39. The pharmacokinetic (PK) assessment at Week 2 was used for dose adjustment after Week 4. Other Names: • Genz-112638
Placebo Comparator: Placebo Placebo	Drug: Placebo Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Outcome Measures

[See Results Section.]

Eligibility

Minimum Age: 16 Years

Maximum Age:

Gender: Both

Accepts Healthy Volunteers?: No

Criteria: Inclusion Criteria:

- The participant (and/or their parent/legal guardian) is willing and able to provide signed informed consent prior to any study-related procedures to be performed
- The participant is at least 16 years old at the time of randomization
- The participant has a confirmed diagnosis of Gaucher disease Type 1
- Female participants of childbearing potential must have a documented negative pregnancy test prior to dosing. In addition all female participants of childbearing potential must use a medically accepted form of contraception throughout the study

Exclusion Criteria:

- The participant has had a partial or total splenectomy
- The participant has received pharmacological chaperones or miglustat within 6 months prior to randomization
- The participant has received enzyme replacement therapy within 9 months prior to randomization
- The participant has Type 2 or 3 Gaucher disease or is suspected of having Type 3 Gaucher disease
- The participant has any clinically significant disease, other than Gaucher disease, including cardiovascular, renal, hepatic, gastrointestinal (GI), pulmonary, neurologic, endocrine, metabolic, (for example, hypokalemia, hypomagnesemia), or psychiatric disease, other medical conditions, or serious intercurrent illness that may confound the study results, or, on the opinion of the investigator, may preclude participation in the study

- The participant has tested positive for the human immunodeficiency virus (HIV) antibody, Hepatitis C antibody, or Hepatitis B surface antigen
- The participant has received an investigational product within 30 days prior to randomization
- The participant is pregnant or lactating

Contacts/Locations

Study Officials: Medical Monitor
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References

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Links:

Study Results

Participant Flow

Pre-Assignment Details	A total of 72 participants were screened of which 32 participants were screen failure. A total of 40 participants were enrolled in this study.

Reporting Groups

	Description
Eliglustat	Eliglustat tartrate capsule as a single 50 milligram (mg) dose on Day 1 followed by eliglustat tartrate 50 mg capsule twice daily (BID) from Day 2 to Week 4, and then either eliglustat tartrate 50 mg capsule BID (in participants who had a Genz-99067 [active moiety of eliglustat tartrate in plasma] trough plasma concentration greater than or equal to [>=] 5 nanogram per milliliter [ng/mL]) or eliglustat tartrate 100 mg capsule BID (in participants who had a Genz-99067 trough plasma concentration less than [<] 5 ng/mL), up to Week 39. The pharmacokinetic (PK) assessment at Week 2 was used for dose adjustment after Week 4.
Placebo	Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Overall Study

	Eliglustat	Placebo
Started	20	20
Completed	19	20
Not Completed	1	0
Withdrawal by Subject	1	0

Baseline Characteristics

Analysis Population Description

Full analysis set included all participants who signed informed consent and received at least one dose of study drug.

Reporting Groups

	Description
Eliglustat	Eliglustat tartrate 50 mg capsule BID orally from Day 1 to Week 4, followed by eliglustat tartrate 50 mg or 100 mg capsule BID orally up to Week 52.

	Description
Placebo	Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Baseline Measures

	Eliglustat	Placebo	Total
Number of Participants	20	20	40
Age, Continuous [units: years] Mean (Standard Deviation)	31.6 (11.55)	32.1 (11.26)	31.8 (11.26)
Gender, Male/Female [units: participants]			
Female	12	8	20
Male	8	12	20
Race/Ethnicity, Customized [units: participants]			
Race: White	19	20	39
Race: Asian	1	0	1
Ethnicity: Not Hispanic or Latino	18	20	38
Ethnicity: Hispanic or Latino	2	0	2
Body Mass Index (BMI) [1] [units: kilogram per square meter (kg/m^2)] Mean (Standard Deviation)	23.3 (2.74)	23.4 (3.54)	23.4 (3.13)
Weight [units: kilogram (kg)] Mean (Standard Deviation)	64.8 (11.74)	68.6 (17.17)	66.7 (14.65)
Height [units: centimeter (cm)] Mean (Standard Deviation)	166.2 (9.91)	170.0 (12.02)	168.1 (11.05)

[1] BMI was calculated as ([weight in kg] divided by [height in cm multiplied by 0.01]^2).

Outcome Measures

1. Primary Outcome Measure:

Measure Title	Percent Change From Baseline in Spleen Volume (in Multiples of Normal [MN]) at Week 39 of the Primary Analysis Period With Eliglustat Tartrate Treatment as Compared to Placebo
Measure Description	Percent change in spleen volume = ([spleen volume at Week 39 minus spleen volume at baseline] divided by [spleen volume at baseline]) multiplied by 100, where all volumes are in MN.
Time Frame	Baseline, Week 39
Safety Issue?	No

Analysis Population Description

Full analysis set included all participants who signed informed consent and received at least one dose of study drug.

Reporting Groups

	Description
Eliglustat	Eliglustat tartrate 50 mg capsule BID orally from Day 1 to Week 4, followed by eliglustat tartrate 50 mg or 100 mg capsule BID orally up to Week 52.
Placebo	Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Measured Values

	Eliglustat	Placebo
Number of Participants Analyzed	20	20
Percent Change From Baseline in Spleen Volume (in Multiples of Normal [MN]) at Week 39 of the Primary Analysis Period With Eliglustat Tartrate Treatment as Compared to Placebo [units: percent change] Least Squares Mean (Standard Error)	-27.77 (2.37)	2.26 (2.37)

Statistical Analysis 1 for Percent Change From Baseline in Spleen Volume (in Multiples of Normal [MN]) at Week 39 of the Primary Analysis Period With Eliglustat Tartrate Treatment as Compared to Placebo

Statistical Analysis Overview	Comparison Groups	Eliglustat, Placebo

	Comments	Analysis was performed using analysis of covariance (ANCOVA) model fitted with treatment and baseline spleen severity (low spleen severity: spleen volume less than or equal to [<=] 20 multiples of normal spleen volume, high spleen severity: spleen volume greater than (>) 20 multiples of normal spleen volume).
	Non-Inferiority or Equivalence Analysis?	No
	Comments	[Not specified]

Statistical Test of Hypothesis	P-Value	<0.0001
	Comments	[Not specified]
	Method	ANCOVA
	Comments	[Not specified]

Method of Estimation	Estimation Parameter	Other [Least Squares Mean Difference]
	Estimated Value	-30.03
	Confidence Interval	(2-Sided) 95% -36.82 to -23.24
	Parameter Dispersion	Type: Standard Error of the mean Value: 3.35
	Estimation Comments	[Not specified]

2. Secondary Outcome Measure:

Measure Title	Hemoglobin Level
Measure Description	
Time Frame	Baseline
Safety Issue?	No

Analysis Population Description

Full analysis set included all participants who signed informed consent and received at least one dose of study drug.

Reporting Groups

	Description
Eliglustat	Eliglustat tartrate 50 mg capsule BID orally from Day 1 to Week 4, followed by eliglustat tartrate 50 mg or 100 mg capsule BID orally up to Week 52.

	Description
Placebo	Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Measured Values

	Eliglustat	Placebo
Number of Participants Analyzed	20	20
Hemoglobin Level [units: gram per deciliter (g/dL)] Mean (Standard Deviation)	12.05 (1.816)	12.75 (1.629)

3. Secondary Outcome Measure:

Measure Title	Absolute Change From Baseline in Hemoglobin Level at Week 39
Measure Description	Absolute change = hemoglobin level at Week 39 minus hemoglobin level at baseline.
Time Frame	Baseline, Week 39
Safety Issue?	No

Analysis Population Description

Full analysis set included all participants who signed informed consent and received at least one dose of study drug.

Reporting Groups

	Description
Eliglustat	Eliglustat tartrate 50 mg capsule BID orally from Day 1 to Week 4, followed by eliglustat tartrate 50 mg or 100 mg capsule BID orally up to Week 52.
Placebo	Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Measured Values

	Eliglustat	Placebo
Number of Participants Analyzed	20	20
Absolute Change From Baseline in Hemoglobin Level at Week 39 [units: g/dL] Least Squares Mean (Standard Error)	0.69 (0.23)	-0.54 (0.23)

4. Secondary Outcome Measure:

Measure Title	Percent Change From Baseline in Liver Volume (in MN) at Week 39
Measure Description	Percent change in liver volume = ([liver volume at Week 39 minus liver volume at baseline] divided by [liver volume at baseline]) multiplied by 100, where all volumes are in MN.
Time Frame	Baseline, Week 39
Safety Issue?	No

Analysis Population Description

Full analysis set included all participants who signed informed consent and received at least one dose of study drug.

Reporting Groups

	Description
Eliglustat	Eliglustat tartrate 50 mg capsule BID orally from Day 1 to Week 4, followed by eliglustat tartrate 50 mg or 100 mg capsule BID orally up to Week 52.
Placebo	Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Measured Values

	Eliglustat	Placebo
Number of Participants Analyzed	20	20
Percent Change From Baseline in Liver Volume (in MN) at Week 39 [units: percent change] Least Squares Mean (Standard Error)	-5.20 (1.64)	1.44 (1.64)

5. Secondary Outcome Measure:

Measure Title	Percent Change From Baseline in Platelet Counts at Week 39
Measure Description	Percent change in platelet count = ([platelet count at Week 39 minus platelet count at baseline] divided by [platelet count at baseline]) multiplied by 100.
Time Frame	Baseline, Week 39
Safety Issue?	No

Analysis Population Description

Full analysis set included all participants who signed informed consent and received at least one dose of study drug.

Reporting Groups

	Description
Eliglustat	Eliglustat tartrate 50 mg capsule BID orally from Day 1 to Week 4, followed by eliglustat tartrate 50 mg or 100 mg capsule BID orally up to Week 52.
Placebo	Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Measured Values

	Eliglustat	Placebo
Number of Participants Analyzed	20	20
Percent Change From Baseline in Platelet Counts at Week 39 [units: percent change] Least Squares Mean (Standard Error)	32.00 (5.95)	-9.06 (5.95)

Reported Adverse Events

Time Frame	From signature of informed consent up to 30-37 days after the last dose of treatment (last dose = up to Week 52)
Additional Description	Safety set included all participants who received at least 1 dose of study drug (Eliglustat or placebo). In the event a single participant experienced both serious and non-serious forms of same adverse events (AE), individual was included in numerator (number of participants affected) of each AE table.

Reporting Groups

	Description
Eliglustat	Eliglustat tartrate 50 mg capsule BID orally from Day 1 to Week 4, followed by eliglustat tartrate 50 mg or 100 mg capsule BID orally up to Week 52.
Placebo	Matching placebo capsule once daily on Day 1 followed by matching placebo capsule BID from Day 2 through Week 39.

Serious Adverse Events

	Eliglustat	Placebo
	Affected/At Risk (%)	Affected/At Risk (%)
Total	0/20 (0%)	0/20 (0%)

Other Adverse Events

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

	Eliglustat	Placebo
	Affected/At Risk (%)	Affected/At Risk (%)
Total	18/20 (90%)	14/20 (70%)
Blood and lymphatic system disorders		
Anaemia [A] †	1/20 (5%)	0/20 (0%)
Splenic haemorrhage [A] †	1/20 (5%)	0/20 (0%)
Cardiac disorders		
Palpitations [A] †	0/20 (0%)	1/20 (5%)
Tachycardia [A] †	1/20 (5%)	0/20 (0%)
Ventricular tachycardia [A] †	0/20 (0%)	1/20 (5%)
Ear and labyrinth disorders		
Vertigo [A] †	1/20 (5%)	0/20 (0%)
Eye disorders		
Eye irritation [A] †	1/20 (5%)	0/20 (0%)
Vision blurred [A] †	0/20 (0%)	1/20 (5%)
Vitreous detachment [A] †	1/20 (5%)	0/20 (0%)
Gastrointestinal disorders		
Abdominal distension [A] †	0/20 (0%)	1/20 (5%)
Abdominal pain [A] †	1/20 (5%)	2/20 (10%)
Abdominal pain upper [A] †	0/20 (0%)	1/20 (5%)

	Eliglustat	Placebo
	Affected/At Risk (%)	Affected/At Risk (%)
Anal pruritus [A] †	1/20 (5%)	0/20 (0%)
Diarrhoea [A] †	3/20 (15%)	4/20 (20%)
Dyspepsia [A] †	1/20 (5%)	0/20 (0%)
Flatulence [A] †	2/20 (10%)	1/20 (5%)
Gastritis [A] †	1/20 (5%)	0/20 (0%)
Gastrointestinal disorder [A] †	0/20 (0%)	1/20 (5%)
Nausea [A] †	2/20 (10%)	1/20 (5%)
Tooth impacted [A] †	1/20 (5%)	0/20 (0%)
Toothache [A] †	1/20 (5%)	3/20 (15%)
Vomiting [A] †	1/20 (5%)	2/20 (10%)
General disorders		
Asthenia [A] †	1/20 (5%)	1/20 (5%)
Chest pain [A] †	1/20 (5%)	1/20 (5%)
Fatigue [A] †	1/20 (5%)	2/20 (10%)
Influenza like illness [A] †	1/20 (5%)	1/20 (5%)
Nodule [A] †	0/20 (0%)	1/20 (5%)
Oedema peripheral [A] †	1/20 (5%)	0/20 (0%)
Pyrexia [A] †	2/20 (10%)	0/20 (0%)
Immune system disorders		
Drug hypersensitivity [A] †	1/20 (5%)	0/20 (0%)
Seasonal allergy [A] †	1/20 (5%)	0/20 (0%)
Infections and infestations		

	Eliglustat	Placebo
	Affected/At Risk (%)	Affected/At Risk (%)
Bronchitis [A] †	1/20 (5%)	0/20 (0%)
Ear infection [A] †	0/20 (0%)	1/20 (5%)
Influenza [A] †	0/20 (0%)	2/20 (10%)
Localised infection [A] †	1/20 (5%)	0/20 (0%)
Nasopharyngitis [A] †	3/20 (15%)	0/20 (0%)
Oral fungal infection [A] †	1/20 (5%)	0/20 (0%)
Oral herpes [A] †	1/20 (5%)	0/20 (0%)
Paronychia [A] †	0/20 (0%)	1/20 (5%)
Pneumonia [A] †	1/20 (5%)	0/20 (0%)
Sinusitis [A] †	2/20 (10%)	1/20 (5%)
Staphylococcal skin infection [A] †	0/20 (0%)	1/20 (5%)
Tonsillitis [A] †	1/20 (5%)	0/20 (0%)
Tonsillitis streptococcal [A] †	0/20 (0%)	1/20 (5%)
Upper respiratory tract infection [A] †	1/20 (5%)	4/20 (20%)
Urinary tract infection [A] †	1/20 (5%)	1/20 (5%)
Viral infection [A] †	1/20 (5%)	0/20 (0%)
Vulvovaginal mycotic infection [A] †	0/20 (0%)	1/20 (5%)
Injury, poisoning and procedural complications		
Contusion [A] †	2/20 (10%)	3/20 (15%)
Fall [A] †	0/20 (0%)	1/20 (5%)
Ligament sprain [A] †	0/20 (0%)	1/20 (5%)
Muscle strain [A] †	1/20 (5%)	0/20 (0%)

	Eliglustat	Placebo
	Affected/At Risk (%)	Affected/At Risk (%)
Procedural pain [A] †	1/20 (5%)	0/20 (0%)
Investigations		
Amino acid level increased [A] †	1/20 (5%)	0/20 (0%)
Bone density decreased [A] †	0/20 (0%)	1/20 (5%)
Gastric pH decreased [A] †	1/20 (5%)	0/20 (0%)
Nuclear magnetic resonance imaging abnormal [A] †	0/20 (0%)	1/20 (5%)
Platelet count decreased [A] †	1/20 (5%)	0/20 (0%)
Metabolism and nutrition disorders		
Decreased appetite [A] †	1/20 (5%)	1/20 (5%)
Hyperhomocysteinaemia [A] †	1/20 (5%)	0/20 (0%)
Hypophosphataemia [A] †	0/20 (0%)	1/20 (5%)
Vitamin B12 deficiency [A] †	1/20 (5%)	1/20 (5%)
Musculoskeletal and connective tissue disorders		
Arthralgia [A] †	9/20 (45%)	2/20 (10%)
Back pain [A] †	0/20 (0%)	1/20 (5%)
Bone pain [A] †	1/20 (5%)	1/20 (5%)
Flank pain [A] †	0/20 (0%)	1/20 (5%)
Musculoskeletal pain [A] †	1/20 (5%)	1/20 (5%)
Osteoporosis [A] †	0/20 (0%)	1/20 (5%)
Pain in extremity [A] †	0/20 (0%)	1/20 (5%)
Sensation of heaviness [A] †	1/20 (5%)	0/20 (0%)
Nervous system disorders		

	Eliglustat	Placebo
	Affected/At Risk (%)	Affected/At Risk (%)
Amnesia [A] †	0/20 (0%)	1/20 (5%)
Balance disorder [A] †	1/20 (5%)	1/20 (5%)
Dizziness [A] †	1/20 (5%)	2/20 (10%)
Headache [A] †	8/20 (40%)	6/20 (30%)
Hypoglycaemic seizure [A] †	1/20 (5%)	0/20 (0%)
Migraine [A] †	2/20 (10%)	0/20 (0%)
Paraesthesia [A] †	1/20 (5%)	0/20 (0%)
Sinus headache [A] †	1/20 (5%)	0/20 (0%)
Tension headache [A] †	1/20 (5%)	0/20 (0%)
Psychiatric disorders		
Anxiety [A] †	1/20 (5%)	0/20 (0%)
Renal and urinary disorders		
Dysuria [A] †	1/20 (5%)	0/20 (0%)
Haematuria [A] †	1/20 (5%)	1/20 (5%)
Reproductive system and breast disorders		
Dysmenorrhoea [A] †	1/20 (5%)	0/20 (0%)
Menorrhagia [A] †	1/20 (5%)	0/20 (0%)
Menstruation irregular [A] †	0/20 (0%)	1/20 (5%)
Uterine polyp [A] †	1/20 (5%)	0/20 (0%)
Respiratory, thoracic and mediastinal disorders		
Cough [A] †	0/20 (0%)	2/20 (10%)
Dyspnoea [A] †	0/20 (0%)	1/20 (5%)
Epistaxis [A] †	1/20 (5%)	1/20 (5%)

	Eliglustat	Placebo
	Affected/At Risk (%)	Affected/At Risk (%)
Nasal congestion [A] †	1/20 (5%)	0/20 (0%)
Nasal dryness [A] †	1/20 (5%)	0/20 (0%)
Nasal obstruction [A] †	2/20 (10%)	0/20 (0%)
Nasal polyps [A] †	1/20 (5%)	0/20 (0%)
Oropharyngeal pain [A] †	2/20 (10%)	1/20 (5%)
Skin and subcutaneous tissue disorders		
Acne [A] †	1/20 (5%)	1/20 (5%)
Dermatitis contact [A] †	1/20 (5%)	0/20 (0%)
Eczema [A] †	0/20 (0%)	1/20 (5%)
Pruritus [A] †	0/20 (0%)	2/20 (10%)
Skin lesion [A] †	1/20 (5%)	0/20 (0%)
Urticaria [A] †	0/20 (0%)	1/20 (5%)
Vascular disorders		
Flushing [A] †	1/20 (5%)	0/20 (0%)
Haematoma [A] †	1/20 (5%)	0/20 (0%)

† Indicates events were collected by systematic assessment.
A Term from vocabulary, MedDRA 15.0

Limitations and Caveats

The results include data up to the end of double-blind primary analysis period (Week 39).

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.

If no publication has occurred within 12 months of the completion of the study, the Investigator shall have the right to publish/present independently the results of the study. The Investigator shall provide the Sponsor with a copy of any such presentation/publication for comment at least 30 days before any presentation/submission for publication. If requested by the Sponsor, any presentation/submission shall be delayed up to 90 days, to allow the Sponsor to preserve its proprietary rights.

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