Extension Study of Nusinersen (BIIB058) in Participants With Spinal Muscular Atrophy Who Previously Participated in a Study With Nusinersen

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Main Information

Primary registry identifying number
LBCTR2022105117

Protocol number
232SM302

MOH registration number

Study registered at the country of origin
Yes

Study registered at the country of origin: Specify

Type of registration
Prospective

Type of registration: Justify
N/A

Date of registration in national regulatory agency
18/10/2022

Primary sponsor
Biogen MA Inc.

Primary sponsor: Country of origin
United States of America

Date of registration in primary registry
09/01/2023

Date of registration in national regulatory agency
18/10/2022

Public title
Extension Study of Nusinersen (BIIB058) in Participants With Spinal Muscular Atrophy Who Previously Participated in a Study With Nusinersen

Acronym

Scientific title
A Long-Term Extension Study of Nusinersen (BIIB058) Administered at Higher Doses in Participants With Spinal Muscular Atrophy Who Previously Participated in an Investigational Study With Nusinersen

Acronym

Brief summary of the study: English
The primary objective of this study is to evaluate the long-term safety and tolerability of nusinersen administered intrathecally at higher doses to participants with spinal muscular atrophy (SMA) who previously participated in study 232SM203 (NCT04089566). The secondary objective of this study is to evaluate the long-term efficacy of nusinersen administered intrathecally at higher doses to participants with SMA who previously participated in study 232SM203 (NCT04089566).

Brief summary of the study: Arabic
الهدف الأساسي من هذه الدراسة هو تقييم السلامة على المدى الطويل وقابلية تحمل nusinersen المعطى داخل القراب بجرعات أعلى للمشاركين المصابين بضمور العضلات الشوكي (SMA) الذين شاركوا سابقًا في دراسة 232SM203 (NCT04089566). الهدف الثانوي لهذه الدراسة هو تقييم الفعالية طويلة المدى لـ nusinersen المعطى داخل القراب بجرعات أعلى للمشاركين مع SMA الذين شاركوا سابقًا في الدراسة 232SM203 (NCT04089566).

Health conditions/problem studied: Specify
Muscular Atrophy, Spinal

Interventions: Specify

Drug: Nusinersen (BIIB058) Administered as specified in the treatment arm

Key inclusion and exclusion criteria: Inclusion criteria

Completed the Day 302 visit in study 232SM203 (NCT04089566) in accordance with the study protocol.

Key inclusion and exclusion criteria: Gender

Both

Key inclusion and exclusion criteria: Specify gender

Key inclusion and exclusion criteria: Age minimum

1

Key inclusion and exclusion criteria: Age maximum

11

Key inclusion and exclusion criteria: Exclusion criteria

- Treatment with another investigational therapy or enrollment in another interventional clinical study after the Day 302 visit in study 232SM203 (NCT04089566).
- Treatment with Zolgensma (all participants) after the Day 302 visit of study 232SM203 (NCT04089566). - Treatment with an approved therapy for SMA (other than Zolgensma) that is inconsistent with protocol requirements for allowed or disallowed concomitant therapies.

NOTE: Other protocol-defined Inclusion/Exclusion criteria may apply.

Type of study

Interventional

Type of intervention

Pharmaceutical

Type of intervention: Specify type

N/A

Trial scope

Other

Trial scope: Specify scope

Study design: Allocation

Non-randomized controlled trial

Study design: Masking

Blinded (masking used)

Study design: Control

N/A

Study phase

3

Study design: Purpose

Treatment

Study design: Specify purpose

N/A

Study design: Assignment

Parallel

Study design: Specify assignment

N/A

IMP has market authorization

Yes, Worldwide

IMP has market authorization: Specify

USA- Europe

Name of IMP

Nusinersen

Year of authorization

2016

Month of authorization

12

Type of IMP

Others

Pharmaceutical class

Antisense oligonucleotide inhibitors

Therapeutic indication

Muscular Atrophy, Spinal

Therapeutic benefit

People with Spinal Muscular Atrophy have an absence of survival of motor neuron (SMN) proteins; nusinersen is an antisense oligonucleotide that increases production of SMN leading to an improvement of their condition.

Study model

N/A

Study model: Explain model

N/A

Study model: Specify model

N/A

Time perspective

N/A

Time perspective: Explain time perspective

N/A

Time perspective: Specify perspective

N/A

Target follow-up duration

Target follow-up duration: Unit

Number of groups/cohorts

Biospecimen retention

Samples with DNA**

Biospecimen description

Plasma, urine, and CSF samples will be collected using standardized procedures. Sample volume will be determined by weight of the patient using standardized volume limitations. Biological samples will be analyzed for PK and Biomarker concentrations.

Target sample size

5

Actual enrollment target size

2

Date of first enrollment: Type

Anticipated

Date of first enrollment: Date

15/01/2023

Date of study closure: Type

Anticipated

Date of study closure: Date

30/09/2027

Recruitment status

Recruiting

Recruitment status: Specify

Date of completion

IPD sharing statement plan

Yes

IPD sharing statement description

In accordance with Biogen's Clinical Trial Transparency and Data Sharing Policy on http://clinicalresearch.biogen.com/

Additional data URL

https://vivli.org/

Admin comments

Trial status

Approved

Secondary Identifying Numbers

Full name of issuing authority	Secondary identifying number
ClinicalTrials.gov	NCT04729907
EudraCT	2020-004708-32

Sources of Monetary or Material Support

Name
Biogen

Secondary Sponsors

Name
N/A

Contact for Public/Scientific Queries

Contact type	Contact full name	Address	Country	Telephone	Email	Affiliation
Public	Dr. Hicham Mansour	Beirut	Lebanon	-	hicham.mansour@gmail.com	SGUMC
Scientific	Medical Director	Innovation House, 70 Norden Road, Maidenhead SL6 4AY	United Kingdom	-	Clinicaltrials@biogen.com	Biogen Idec Research Limited

Centers/Hospitals Involved in the Study

Center/Hospital name	Name of principles investigator	Principles investigator speciality	Ethical approval
Saint George University Medical Center	Dr. Hicham Mansour	Pediatric Neurology and Metabolic Diseases	Approved

Ethics Review

Ethics approval obtained	Approval date	Contact name	Contact email	Contact phone
Saint George Hospital University Medical Center	10/02/2022	Dr. Michel Daher	-	+961 1 566780

Countries of Recruitment

Name
Lebanon
United States of America
Brazil
Estonia
Germany
Japan
Taiwan
Canada
Russian Federation
Poland
Spain

Health Conditions or Problems Studied

Condition	Code	Keyword
Spinal Muscular Atrophy	Spinal muscular atrophy and related syndromes (G12)	Muscular Atrophy, Spinal, Atrophy Pathological Conditions, Anatomical Neuromuscular Manifestations, Neurologic Manifestations, Nervous System Diseases, Spinal Cord Diseases, Central Nervous System Diseases, Motor Neuron Disease, Neurodegenerative Diseases, Neuromuscular Diseases

Interventions

Intervention	Description	Keyword
Drug	Nusinersen (BIIB058)	-

Primary Outcomes

Name	Time Points	Measure
To evaluate the long-term safety and tolerability of nusinersen	Up to Day 1081	Number of Participants With Adverse Events (AEs) and Serious Adverse Events (SAEs)
To evaluate the long-term safety and tolerability of nusinersen	Up to Day 1081	Change from Baseline in Growth Parameters
To evaluate the long-term safety and tolerability of nusinersen	Up to Day 1081	Number of Participants With Shifts from Baseline in Clinical Laboratory Parameters
To evaluate the long-term safety and tolerability of nusinersen	Up to Day 1081	Number of Participants With Shifts from Baseline in Electrocardiogram (ECG)
To evaluate the long-term safety and tolerability of nusinersen	Up to Day 1081	Number of Participants With Shifts from Baseline in Vital Signs
To evaluate the long-term safety and tolerability of nusinersen	Up to Day 961	Change from Baseline in Activated Partial Thromboplastin Time (aPTT)
To evaluate the long-term safety and tolerability of nusinersen	Up to Day 961	Change from Baseline in Prothrombin Time (PT)
To evaluate the long-term safety and tolerability of nusinersen	Up to Day 961	Change from Baseline in International Normalized Ratio (INR)

Key Secondary Outcomes

Name	Time Points	Measure
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Total Number of New World Health Organization (WHO) Motor Milestones
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Number of Participants Who Used Respiratory Support, by Type
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Number of Hours Per Day of Respiratory Support
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Number of Days That Respiratory Support Is Used
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Time to Death (Overall Survival)
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Change from Baseline in Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) Total Score
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Change from Baseline in Hammersmith Infant Neurological Examination (HINE) Section 2 Motor Milestones
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Percentage of HINE Section 2 Motor Milestone Responders
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Percentage of Time Spent on Ventilation
To evaluate the long-term efficacy of nusinersen	Up to Day 1081	Time to Death or Permanent Ventilation

Trial Results

Summary results

Study results globally

Date of posting of results summaries

Date of first journal publication of results

Results URL link

Baseline characteristics

Participant flow

Adverse events

Outcome measures

URL to protocol files