A Natural History Study of Exocrine Pancreatic Function in Infants with Cystic Fibrosis Less Than 12 Months of Age

Published: 02-12-2024
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To characterize the natural history of exocrine pancreatic function as measured by FE-1 in infants with CF

Ethical review	Approved WMO
Status	Will not start
Health condition type	Respiratory disorders congenital
Study type	Observational invasive

Summary

ID

NL-OMON57135

Source
ToetsingOnline

Brief title
Natural History Study of Exocrine Pancreatic Function in Infants With CF

Condition

- Respiratory disorders congenital
- Exocrine pancreas conditions
- Congenital respiratory tract disorders

Synonym
Exocrine pancreatic insufficiency (EPI)

Research involving
Human

Sponsors and support

Primary sponsor: Vertex Pharmaceuticals
Source(s) of monetary or material Support: Vertex Pharmaceuticals Incorporated

Intervention

Keyword: Cystic Fibrosis, Exocrine Pancreatic Function, Natural History Study

Outcome measures

Primary outcome

Proportion of participants with FE-1 >=200 µg/g over time

Secondary outcome

FE-1 level over time

Study description

Background summary

This study is being done to learn more about the pancreas (an organ that helps digest food) and the intestines in infants with CF who are not treated with a Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) modulator. Caregivers will collect weekly stool samples for testing of a pancreatic enzyme called fecal elastase and a marker of inflammation of the intestines called calprotectin.

Study objective

To characterize the natural history of exocrine pancreatic function as measured by
FE-1 in infants with CF <12 months of age

Study design

This is a hybrid decentralized study to characterize the natural history of exocrine
pancreatic function by measuring FE-1 in infants with CF. Fecal calprotectin (a biomarker of gastrointestinal inflammation) will also be assessed. In the optional

The day of enrollment into this study will be referred to as the *index date*. Data will be collected retrospectively from medical records during the Pre-enrollment
Period, which is defined from date of birth to index date. Data will be collected
prospectively during the Measurement Period, which is defined from index date to
approximately when the participant turns 12 months of age. FE-1 and fecal calprotectin levels will be assessed in fecal samples collected during the Measurement Period.

Intervention

NA

Study burden and risks

Stool sample: there are no known risks or discomforts from the stool sample collection

Confidentiality: There is a possibility that your name or other personal information could be seen by an unauthorized person.

Contacts

Public
Vertex Pharmaceuticals

Van Swietenlaan 6
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NL
Scientific
Vertex Pharmaceuticals

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Babies and toddlers (28 days-23 months)
Newborns

Inclusion criteria

1. Participant*s legally appointed and authorized representative (e.g., parent or legal guardian)
will sign and date an informed consent form (ICF).
2. Male or female participants with CF <6 months of age at the index date.
3. Participant is not eligible to receive commercial Kalydeco* (based on local product labels)
and is not receiving Kalydeco or any other CFTR modulator.
4. As judged by the investigator, the parent or legal guardian must be able to understand
protocol requirements, restrictions, and instructions, and the parent or legal guardian should
be able to ensure that the participant will comply with and is likely to complete the study as
planned.

Exclusion criteria

1. History of any illness or any clinical condition that, in the opinion of the investigator, might
either confound the results of the study or impact participant*s ability to participate.
2. Ongoing or any prior participation in an investigational drug study.
Note: Ongoing participation in a noninterventional study (including observational
studies) is permitted.
3. Participant whose mother took any CFTR modulator while pregnant with the participant, or
who has any history of exposure to a CFTR modulator (e.g., through consumption of breast
milk from a mother on a CFTR modulator or other means).
4. A close relative of the participant is the investigator or a

subinvestigator, research assistant,
study coordinator, or other staff directly involved with the conduct of the study.

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Other

Recruitment

NL

Recruitment status:	Will not start
Enrollment:	4
Type:	Anticipated

Ethics review

Approved WMO

Date:	02-12-2024
Application type:	First submission
Review commission:	BEBO: Stichting Beoordeling Ethiek Bio-Medisch Onderzoek (Assen)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL86940.056.24
Other	To be determined

Study results

Summary results
Trial never started