Delineating a full phenotype of health, neurodevelopment and adaptive functioning in children and young adults with Fetal Valproate Spectrum Disorder.

First published: 26/01/2022

Last updated: 23/04/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS45205

Study ID

47518

DARWIN EU® study

No

Study countries

Australia

Ireland

New Zealand

United Kingdom

Study description

Exposure in the womb to the antiseizure medication sodium valproate (VPA) is associated with an increased risk of altered fetal development leading to a range of congenital anomalies and neurodevelopmental difficulties (Fetal Valproate Spectrum Disorder (ICD-11 LD2F.03). There is limited data available regarding the health and neurodevelopment of children and young people with Fetal Valproate Spectrum Disorder (FVSD). In younger and school age children information is extrapolated from studies investigating the risks associated with VPA exposure, but studies into older children and young people with fetal VPA exposure are extremely limited. Further, there is almost no documented evidence about health and neurodevelopmental outcomes for teenagers and young adults with the condition, particularly from the second decade of life onwards. This project aims to ascertain parent views regarding the health and neurodevelopmental difficulties experienced by children and young people with FVSD as they get older. This is a preliminary study and utilises both standardised and validated questionnaires, and a set of bespoke questions specific to later outcomes that have been developed through expert collaboration and engagement with key charities who support families who have a child with FVSD. While the findings will themselves provide key insights into the wider phenotype of FVSD in older children and young adults, the results will also aid the design of future, more detailed clinical studies regarding health and neurodevelopment in older children and young adults with FVSD, which in turn, will provide the required information to enable optimal diagnosis and intervention through clinical services.

Study status

Ongoing

Research institutions and networks

Institutions

University of Manchester

United Kingdom

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Educational Institution

Contact details

Study institution contact

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Primary lead investigator

Rebecca Bromley

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/04/2019

Actual: 01/04/2019

Study start date
Planned: 14/02/2022

Actual: 18/02/2022

Data analysis start date
Planned: 01/06/2022

Actual: 01/09/2022

Date of final study report
Planned: 19/12/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

IMI ConcePTION, Research/Clinical Salary

Study protocol

EUPAS45205-45352.pdf (774.81 KB)

ConcePTION WP2 Demonstration 3. Protocol Version 2. 24.01.22.pdf (768.53 KB)

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Other

If ‘other’, further details on the scope of the study

Phenotyping Study

Main study objective:

To ascertain maternal views regarding the health, neurodevelopment and adaptive functioning of children and young adults with Fetal Valproate Spectrum Disorder in order to establish the phenotype of FVSD in older children and young adults.

Study Design

Non-interventional study design

Cohort

Cross-sectional

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(N03AG01) valproic acid

valproic acid

Medical condition to be studied

Foetal anticonvulsant syndrome

Additional medical condition(s)

Fetal Valproate Spectrum Disorder

Population studied

Age groups

- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- Adults (18 to < 46 years)

Estimated number of subjects

114

Study design details

Outcomes

Parent reported cognitive development as measured by the Patient Reported Outcome Measurement Information System (PROMIS) Cognitive Function Item Bank (custom short-form). The following outcomes will also be explored: - Physical Health - Neurodevelopmental disorders - Service utilization - Academic Functioning & Educational Outcomes - Social Development - Emotional & Behavioral Development - Sensory Issues - Employment (16+ only) - Independence & Daily Living

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Data analysis plan

Data will be explored and described in terms of means, frequencies and percentages for the FVSD and comparison groups. This data will be inspected to develop a preliminary conceptualization of the FVSD phenotype. Differences within the FVSD group by age, sex and dose will also be explored. Appropriate statistical tests (e.g. t-test and Chi-square) will then be used to examine differences between the group with FVSD and the control group and, should large enough numbers be obtained, analysis adjusting for confounder and moderating and/or mediating factors will be undertaken (e.g. multiple linear and logistic regression analyses). Data from each age group will be pooled for analysis where appropriate and group differences will be explored using either total/mean scores (for scales measuring an underlying latent trait) or frequencies (for checklists of directly observable variables).

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

This study has been awarded the ENCePP seal

Conflicts of interest of investigators

EUPAS45205-45354.pdf (164.98 KB)

Composition of steering group and observers

EUPAS45205 Composition of Steering Group and Observers.pdf (53.51 KB)

EUPAS45205-45486.pdf (53.51 KB)

Data sources

Data sources (types)

Other

Data sources (types), other

This study will acquire new data directly from parents of children and young people with FVSD. The study will be promoted through collaboration with Charities who support families of children with Fetal Valproate Spectrum Disorder. This will include international Charities but limited to English speaking countries.

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No