

DEFI-ALPHA - Cohort of Children with Alpha 1 Antitrypsin Deficiency.

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General

Identification

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|--|---|
| Detailed name | Cohort of Children with Alpha 1 Antitrypsin Deficiency. |
| Sign or acronym | DEFI-ALPHA |
| CNIL registration number, number and date of CPP agreement, AFSSAPS (French Health Products Safety Agency) authorisation | CCTIRS n°10.181 (08/04/2010), CNIL n°910279 (DR-2010-328, 29/10/2010) |

General Aspects

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|---------------------|--|
| Medical area | Gastroenterology et hepatology |
| Health determinants | Genetic |
| Keywords | occurrence of complications, ultrasound/Doppler ultrasound, FibroScan, fibrosis test, endoscopy and oesophageal video capsule, platelets, transaminase and gamma-GT, Health episodes, factors, liver transplantation |

Scientific investigator(s) (Contact)

| | |
|----------------------|---|
| Name of the director | Lachaux |
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| Unit | Service d'Hépatologie, Gastroentérologie et Nutrition, |
| Organization | HCL - HOPITAL FEMME-MERE-ENFANTDE LYON |

Collaborations

Participation in projects,
networks and consortia

Yes

Funding

Funding status

Mixed

Details

Laboratoires LFB, PHRC

Governance of the database

Sponsor(s) or organisation(s)
responsible

CHU Lyon (L. Restier)

Organisation status

Public

Additional contact

Name of the contact

MIRON RESTIER

Surname

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Unit

Service d'Hépatologie, Gastroentérologie et
Nutrition,

Organization

HCL - HOPITAL FEMME-MERE-ENFANTDE LYON

Main features

Type of database

Type of database

Study databases

Study databases (details)

Cohort study

Database recruitment is carried
out by an intermediary

A selection of health institutions and services

Database recruitment is carried
out as part of an interventional
study

No

Additional information regarding
sample selection.

Retrospective Other bodies active in creating this
cohort: CHU and CHG, parents' association
(Association ADAAT ALPHA 1 France, French

Database objective

Main objective

General objective: To investigate prognostic hepatic factors in A1AT deficient children. Investigation of factors associated with onset of complications: portal hypertension and its complications, severe liver failure, liver transplantation, abnormalities during respiratory function exploration. Secondary objectives: - To organise an active homogenised cohort follow-up. - To ensure that new cases are recorded. - To create a reference network in order to homogenise treatment.

Inclusion criteria

Children with DA1AT born after 1989, regardless of phenotype. DA1AT is defined as an alpha-1 antitrypsin deficiency lower than 1,1 g/l, and type ZZ or SZ MZ, MS, SS, detected by participating centres (service follow-up, or detected by corresponding neonatal services, family surveys, patient associations or dosage laboratories in the geographical area of participating services).

Population type

Age

Early childhood (2 to 5 years)
Childhood (6 to 13 years)
Adolescence (13 to 18 years)

Population covered

Sick population

Pathology

XI - Diseases of the digestive system

Gender

Male
Woman

Geography area

National

Detail of the geography area

Multicentric cohort throughout France (15 centres)

Data collection

Dates

Date of first collection (YYYY or MM/YYYY)

09/2008

Size of the database

Size of the database (number of individuals)

< 500 individuals

| | |
|--------------------------------------|-----|
| Details of the number of individuals | 180 |
|--------------------------------------|-----|

Data

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|-------------------|-------------------------|
| Database activity | Current data collection |
|-------------------|-------------------------|

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|------------------------|---|
| Type of data collected | Clinical data Paraclinical data Biological data |
|------------------------|---|

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|------------------------|--|
| Clinical data (detail) | Direct physical measures Medical registration |
|------------------------|--|

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|----------------------------|---|
| Paraclinical data (detail) | Biochemistry and genetics, ultrasound, FibroScan, and medical imaging |
|----------------------------|---|

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|--------------------------|------------------|
| Biological data (detail) | Clinical records |
|--------------------------|------------------|

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| Presence of a biobank | Yes |
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|---------------------|-------|
| Contents of biobank | Serum |
|---------------------|-------|

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|----------------------------|------------|
| Details of biobank content | Serum bank |
|----------------------------|------------|

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|---------------------------|--|
| Health parameters studied | Health event/morbidity Health event/mortality |
|---------------------------|--|

Procedures

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| Data collection method | Interview: Direct input Clinical examination: Direct input Biological analysis: Direct input |
|------------------------|--|

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| Participant monitoring | Yes |
|------------------------|-----|

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|-----------------------|---|
| Monitoring procedures | Monitoring by crossing with a medical-administrative database |
|-----------------------|---|

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|---------------------------------------|-----------------------|
| Details on monitoring of participants | Until 18 years of age |
|---------------------------------------|-----------------------|

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|--------------------|---------------------------------------|
| Followed pathology | XI - Diseases of the digestive system |
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|---------------------------------|----|
| Links to administrative sources | No |
|---------------------------------|----|

Promotion and access

Promotion

Link to the document

[Ruiz_et_al-2019-Liver_International.pdf](#)

Description

Pathologies hépatiques en rapport avec le déficit en alpha1-antitrypsine dans une cohorte d'enfants en France

Access

Terms of data access (charter for data provision, format of data, availability delay)

To be decided if data may be used by academic teams Eric Chevet, INSERM U1053, Université Bordeaux Ségalen Access conditions: for European cohort collaboration (Dino Hadzic, nedim.hadzic@kcl.ac.uk), for an adult cohort with DA1AT (Gabriel Thabut : g.thabut@bch.ap-hop-paris.fr) To be decided if data may be used by industrial teams Access for potentially interested LFB laboratories

Access to aggregated data

Access on specific project only

Access to individual data

Access on specific project only