Portal da Atrofia Muscular Espinhal (Spinal Muscular Atrophy Portal)

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Data source | Human | Disease registry | Drug registry

Administrative details

Administrative details

Data source ID

1000000930

Data source acronym

PAME

Data holder

INFARMED – National Authority of Medicines and Health Products, I.P.

Data source type

Disease registry

Drug registry

Main financial support

Funding by own institution

Care setting

Hospital outpatient care

Data source qualification

If the data source has successfully undergone a formal qualification process (e.g., from the EMA, ISO or other certifications), this should be described.

No

Description of the qualification

undefined

Contact details

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Data source regions and languages

Data source countries

Portugal

Data source languages

Portuguese

Data source regions

Região Autónoma da Madeira

Região Autónoma dos Açores

Região Continental

Data source establishment

Data source established

08/03/2019

Data source time span

First collection: 22/03/2019

The date when data started to be collected or extracted.

Data elements collected

The data source contains the following information

Disease information

Does the data source collect information with a focus on a specific disease? This might be a patient registry or other similar initiatives.

Yes

Disease details

Spinal muscular atrophy

Rare diseases

Are rare diseases captured? In the European Union a rare disease is one that affects no more than 5 people in 10,000.

Yes

Pregnancy and/or neonates

Does the data source collect information on pregnant women and/or neonatal subpopulation (under 28 days of age)?

Yes

Hospital admission and/or discharge

No

ICU admission

Is information on intensive care unit admission available?

No

Cause of death

Captured

Cause of death vocabulary

Not coded (Free text)

Prescriptions of medicines

Captured

Prescriptions vocabulary

other

Prescriptions vocabulary, other

Active ingredient(s)

Dispensing of medicines

Not Captured

Advanced therapy medicinal products (ATMP)

Is information on advanced therapy medicinal products included? A medicinal product for human use that is either a gene therapy medicinal product, a somatic cell therapy product or a tissue engineered products as defined in Regulation (EC) No 1394/2007 [Reg (EC) No 1394/2007 Art 1(1)].

Yes

Contraception

Is information on the use of any type of contraception (oral, injectable, devices etc.) available?

No

Indication for use

Does the data source capture information on the therapeutic indication for the use of medicinal products?

Captured

Indication vocabulary

Other

Medical devices

Is information on medicinal devices (e.g., pens, syringes, inhalers) available?

No

Administration of vaccines

No

Procedures

Does the data source capture information on procedures (e.g., diagnostic tests, therapeutic, surgical interventions)?

Captured

Procedures vocabulary

Not coded (Free text)

Healthcare provider

Is information on the person providing healthcare (e.g., physician, pharmacist, specialist) available? The healthcare provider refers to individual health professionals or a health facility organisation licensed to provide health care diagnosis and treatment services including medication, surgery and medical devices.

Yes

Clinical measurements

Is information on clinical measurements (e.g., BMI, blood pressure, height) available?

Yes

Genetic data

Are data related to genotyping, genome sequencing available?

Captured

Biomarker data

Does the data source capture biomarker information? The term “biomarker” refers to a broad subcategory of medical signs (objective indications of medical state observed from outside the patient), which can be measured accurately and reproducibly. For example, haematological assays, infectious disease markers or metabolomic biomarkers.

Not Captured

Patient-reported outcomes

Is information on patient-reported outcomes (e.g., quality of life) available?

No

Patient-generated data

Is patient-generated information (e.g., from wearable devices) available?

No

Units of healthcare utilisation

Are units of healthcare utilisation (e.g., number of visits to GP per year, number of hospital days) available or can they be derived? Units of healthcare utilisation refer to the quantification of the use of services for the purpose of preventing or curing health problems.

No

Unique identifier for persons

Are patients uniquely identified in the data source?

Yes

Diagnostic codes

Not Captured

Medicinal product information

Captured

Medicinal product information collected

Active ingredient(s)

Dosage regime

Dose

Medicinal product vocabulary

Not coded (Free text)

Quality of life measurements

Not Captured

Lifestyle factors

Not Captured

Sociodemographic information

Captured

Sociodemographic information collected

Age

Sex

Quantitative descriptors

Population Qualitative Data

Population age groups

Paediatric Population (< 18 years)

Neonate

Preterm newborn infants (0 – 27 days)

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adult and elderly population (≥18 years)

Adults (18 to < 65 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Elderly (≥ 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated percentage of the population covered by the data source in the catchment area

100% of the patients treated with medicines for spinal muscular atrophy, as the approval for finnancing depends on registration in the Portal.

Data flows and management

Access and validation

Biospecimen access

Are biospecimens available in the data source (e.g., tissue samples)?

No

Access to subject details

Can individual patients/practitioners/practices included in the data source be contacted?

No

Description of data collection

Recording on a dedicated platform by practioners.

Event triggering registration

Event triggering registration of a person in the data source

Disease diagnosis

Event triggering de-registration of a person in the data source

Death

End of treatment

Loss to follow up

Event triggering creation of a record in the data source

Specialist encounter, as defined by the platform rules.

Data source linkage

Linkage

Is the data source described created by the linkage of other data sources (prelinked data source) and/or can the data source be linked to other data source on an ad-hoc basis?

No

Data management specifications that apply for the data source

Possibility of data validation

Can validity of the data in the data source be verified (e.g., access to original medical charts)?

No

Data source preservation

Are records preserved in the data source indefinitely?

Yes

Approval for publication

Is an approval needed for publishing the results of a study using the data source?

No

Common Data Model (CDM) mapping

CDM mapping

Has the data source been converted (ETL-ed) to a common data model?

No