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Czech national registry of myotonic disorders

REaDY

REgistry of muscular **DY**strophy

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Project REaDY

- Start in may 2011
- Data collection about treatment and development of muscle diseases
  - DMD/BMD (Duchenn/Becker muscular dystrophy)
  - SMA (spinal muscular atrophy)
  - MD type 1 a 2 (myotonic dystrophy)

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What are neuromuscular diseases?

- In most cases **rare (orphan) diseases**
- Containe all **autoimumne** and **hereditary** neuromuscular diseases
  - **EU:** < 1 / 2000 people
  - **Japan:** < 1 / 2500 people
  - **USA:** affects fewer than 200,000 people in the US population
- **= not much experience with this diseases**

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Myotonic dystrophy

- The most common muscular dystrophy in adults
- Two types: MD 1 a MD 2
- Recent studies suggest that type 2 could be more common as type 1
- MD 2 has significantly milder progress than MD 1
- Prevalence: 1-5/10 000

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Recent data in Czech Republic

	Pilsen	Prague	Pardubice	Hradec	Brno	Olomouc	Ostrava	Total
MD1	0	49	8	5	17+1	6	2	87
MD2	10	51	10	6	52+3	4	19	152

Source:
•Local registries of Czech neuromuscular centers

•Centre of Molecular Biology Brno (DNA diagnostics)

•2004- 2010

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Target of project REaDY

- **Create and lead** the database of czech and slovak patients with DMD/BMD, SMA and DM
- Place this patients to **internacional studies**
- In the case of a new treatment develop, **speed up a choice** of suitable patients
- Support a muscular dystrophy **research**
- Provide a information about **the newest ways of treatment**

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Rules for a registry working

- Before inclusion to registry a patient sign **the informed consent.** Patient can **withdraw** from the registry anytime.
- The collection of personal information is notify to „**the office for personal data protection“**
- Participation in registry is **voluntary** and there are **no fee** or **taxes**
- Active patients **collaboration**, patients may control their data in on-line registry
- The system is **secure** (in server with system Oracle 9i).
- Project is led by team of **medical specialists**

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Basic attributes of the system

- **User friendly** and easy to understand, work via internet browser
- Data can be set from **any computer** with internet connection and browser
- **No other softwar** to use the registry
- **Only authorized person** may enter the registry (personal login and password)
- Data are **anonymous** – every patient has personal **ID (f.e.: DM-110908M-NS)**

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Basic attributes of the system

- All data transmitions are **coded**
- All data are collected in **central computer** (server with ORACLE 9i)
- All data can be **exported** as a local database only by authorized person
- User may **print or save** filled-out form in MS Excel protocol
- **USER FRIENDLY – SECURE – PERSONAL DATA PROTECT**

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Data sharing

- Each neuromuscular center may see **only own data**
- Sharing is possible only **with assent** of data owner and council of project leaders

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Registry structure

- Created in accordance with international muscular dystrophy registry **TREAT-NMD**
- **Enrollment** – basic information about patient (several groups)
- **Follow-up** – check and describe of update conditions
- **Survival status** – date and cause of death

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Enrollment

- Initials, date of birth, sex, phone, email
- Diagnosis (MD 1, MD 2, other)
- Motor function (Wheelchair use ?, 6-minute walk test)
- MRC scale (muscle weakness: 0-5 points)
- Presence and seriousness of myotonia
- Heart function (US, ECG), lung function (spirometry), presence or surgery of cataracta, ...
- Pain questionnaire, QoL, Depresion scale

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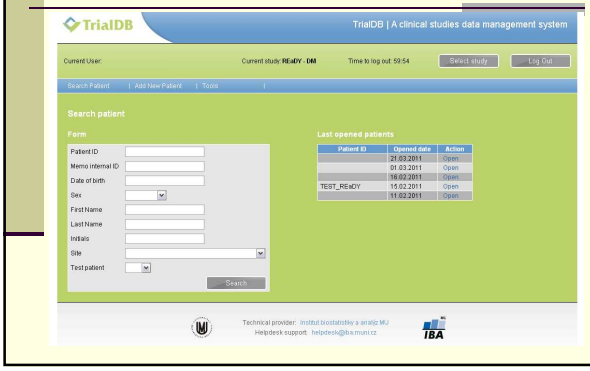
Registry view



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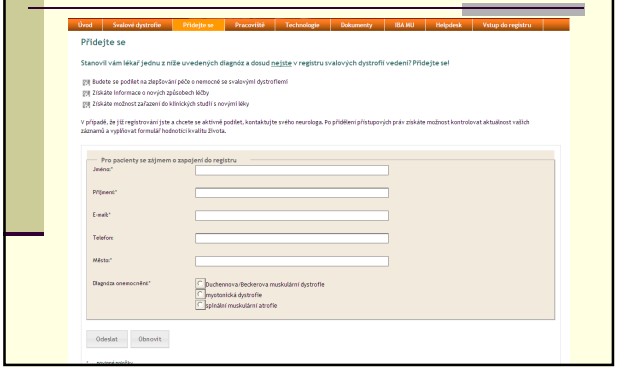
Registry view



THANK YOU FOR YOUR ATTENTION

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