The „Czech way“ to Haemophilia Care

Jan Blatný
Department of Paediatric Haematology
University Hospital Brno & Masaryk University
Brno, CZ

The Marathon

As in any race
• It is NOT enough to start the run!
• Do NOT STOP half way
• Get to the FINISH

• It is similar for the development of haemophilia care

24 years ago – in former Czechoslovakia

• “Iron curtain” was shut down!!
  – (Almost) Impossible to obtain factor concentrates from the world “behind the curtain”
  – FFP, Cryo treatment only
  – Factor VIII consumption far below 1IU/capita/year
• NO home treatment
  – Hospital admission necessary for almost any treatment
• NO prophylaxis
  – Devastation of locomotory apparatus of many haemophiliacs
• Treatment LIMITED in most severe cases
• FRUSTRATING for patients as well as for caregivers
• “Run for haemophilia” was almost impossible!
Velvet revolution!!!

- After the political changes in 1989
  - The "run" could finally "go on"!!!
- Czechoslovakia peacefully divided into two sovereign countries on January 1st 1993.

Demographics of Czech Republic

- With cca 79 000 km²
- Czech Republic is home for over 10 million people
- Almost 1000 persons with haemophilia
  - One fifth of them are children (up to 18 years of age)
- Less than 10 new children with haemophilia born per year

STARTING THE RACE
Starting the race is always a challenge!

- Factor concentrates became available after 1990!
  - “Proper” and efficient “on-demand treatment” started
  - Home treatment introduced to Czech patients
- Followed by short-term secondary prophylaxis
  - After significant bleeds
  - During physiotherapy
  - During and after surgical interventions
  - Total hip replacements became a part of ambitious surgical interventions programme
- To get our patients “back from their wheel-chairs”!!!
- Following the WFH recommendations led to:
  - Building up the haemophilia centres network
  - Further promoting home treatment for all, who needed it

Running further – late 90’s

- Progress in and bettering of haemophilia care
- Improved availability of clotting concentrates
  - Medium-high purity plasma derived concentrates available
- Secondary prophylaxis to all children
  - Major change in the haemophilia care for the country!
  - Regimen similar to “Dutch” chosen at that time
  - Prophylaxis starting after first “few” significant bleeds
  - Lower dosing (median of 24 IU/kg 2-3x per week)
  - Tailored approach (certain severe patients might do well “on-demand”, based more on “phenotype” than solely on “genotype” of particular patient)
  - Treatment for all children, who needed prophylaxis, at affordable costs
  - Major change in the haemophilia care for the country!

Five pillars of the “Bridge over troubled water” our race run over.

- Prophylaxis to ALL kids with severe phenotype
- Home treatment available for ALL
- Keep maximal safety of the treatment
  - “iron-curtain” was uncrossable also for blood born infections including HIV!!!
  - Around 30 HIV+ including 7 children only
- Efficient and Immediate treatment for patients with inhibitors
- Building up the Haemophilia Centres network
HALF WAY BEHIND US

Czech Haemophilia centres network

2+2 x CCC (Paed/Adults)
7 x HTC
Per 10 mil inhabitants in CZ

Current situation and strategy!

- FVIII consumption 4,3 IU/capita/year
- ALL children on primary prophylaxis
  - Commenced not later than during 2nd year of life and/or after 1st bleed
  - Starting gradually with escalating frequency/dose
    - Often “Bremen/Munich regimen” (Kurnik et al., 2009, EPIC study)
    - To reach final dose of 25 IU/kg/3xweekly (HA) or twice per week (HB)
- Young adults tend to continue on prophylaxis
- CVLs used rarely
  - Patients are doing well with peripheral vein access
- Increasing number of patients treated with recombinants
  - Since 2006 treatment of choice for PUPs and MTPs
  - Switch to recombinants offered also to other “eligible” patients
    - Currently about 10% of total FVIII consumption per year
- No signs of increased inhibitors rates so far
Current situation and strategy!

- Promising safety profile
  - 4 HIV+ (including one patient infected as a child)
  - 127 HCV+ (including 5 children)
- Low inhibitors rates (HA)
  - Incidence 6% in age group 0-18 years
  - Total prevalence 2.16%
  - Within paediatric population 4.1%
- ITI treatment of choice in children
  - Over 80% success rate
- By-pass medication (rFVIIa, aPCC) for adults
- Elective surgical interventions including hip and knee arthroplasty do continue in adults
  - To "pay the bill" made by previous inappropriate and insufficient therapy

Current situation and strategy!

- Czech National Haemophilia Programme established
  - Multi disciplinary professional nation-wide initiative
  - All 4CCC + 7HTC involved
  - Regular accreditation of centres
  - Standards & Guidelines formulation
  - Promoting "European principles of haemophilia care"
  - Cooperation with health-care payers and legal authorities
  - Both patients' organizations involved
    - Czech Haemophilia Union (WFH member)
    - Hemojunior (Parents' organization)
- Participating in international projects
  - ESChQoL, EUHASS, EUHANET etc...
- Building up the "PR" for haemophilia together
  - Do not forget about vWD patients...

Czech National Hemophilia Program

2011 CNHP REGISTRY DATA
(ON REPRESENTATIVE POPULATION SAMPLE)
**Age**

Age at diagnosis (years):

- N = 419*
- Mean: 8.1
- Median (min – max): 2 (0 – 73)

Current age (years):

- N = 595
- Mean: 30.0
- Median (min – max): 25 (0 – 89)

25.0% of persons age (years)

* Missing information on year of diagnosis in 176 persons.

**Type and severity of haemophilia II**

Haemophilia A (N=5011)

- Mild (N=223)
- Moderate (N=78)
- Severe (N=200)

Haemophilia B (N=732)

- Mild (N=28)
- Moderate (N=33)
- Severe (N=33)

All N=595

- 27.4%
- 27.4%
- 45.2%
- 44.5%
- 15.6%
- 39.9%

Severity not known in 17 persons with haemophilia A.

Severity not known in 4 persons with haemophilia B.

**Hepatitis**

Hepatitis infection

- Yes (N=157)
- No (N=408)
- Not known (N=30)

All N=595

- 82.2%
- 80%
- 100%

Types of hepatitis:

- Hep A (N=26)
- Hep B (N=62)
- Hep C (N=129)
- Type of hepatitis not specified in 3 persons.
- One person may have recorded more types of hepatitis.
HIV

HIV
Positive (N=4)
Negative (N=460)
Not available (N=131)

All 4 HIV-positive persons are adults.

Data from last annual report of each person.

Frequency of bleeding in 2011

Frequency of bleeding in 2011
Children (N=170)

Frequency of bleeding is missing in 51 children.

Frequency of bleeding in 2011

Frequency of bleeding in 2011

Frequency of bleeding is missing in 94 persons.
Frequency of bleeding in 2011

201 (70%) persons experienced bleeding at least once per year (1121 bleeds were recorded in total). 86 persons recorded no bleed during year 2011. Information on frequency of bleeding is missing in 94 persons.

Location of bleeds in 2011

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WE WILL RUN FURTHER...
What are our “Czechlenges”

- Increase the factor consumption
  - Around 6 IU/capita per year shall be probably sufficient

- Increase the use of recombinants
  - New paediatric recommendations in place since 2006
    - PUPs, MTPs (incl. those on prophylaxis)
    - Pregnant carriers
  - Adult patients?! Introduction of rFIX?! Longer acting concentrates?

- More prophylaxis for (young) adults
  - Our children do become adult and want to maintain high QoL!!!
  - Seniors have other diseases. Why to stress them with haemophilia related risks, if not necessary?

- Enforcing Czech National Haemophilia Programme