




The „Czech way“ to Haemophilia Care




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The Marathon




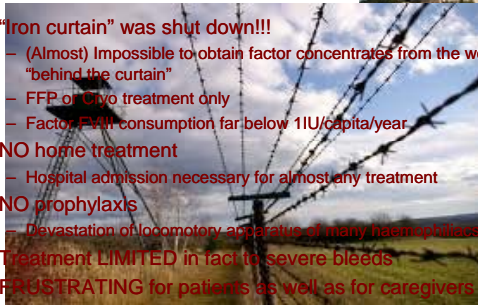
As in any race

- 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 or Cryo treatment only
 - Factor FVIII 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 fact to severe bleeds
- 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!



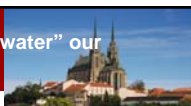
- Factor concentrates became available after 1990!
 - “Proper” and efficient “on-demand treatment” started
 - Home treatment introduced to Czech patients
 - Followed by short-term secondary prophylaxis
 - After significant bleeds
 - During physiotherapy
 - During and after surgical interventions
 - Total 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

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



Česká republika

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" (K 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
 - Excluding transient iFVIII
 - 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

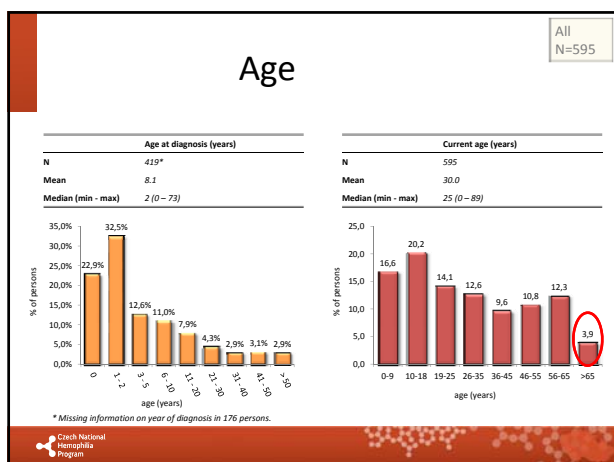
Current situation and strategy!

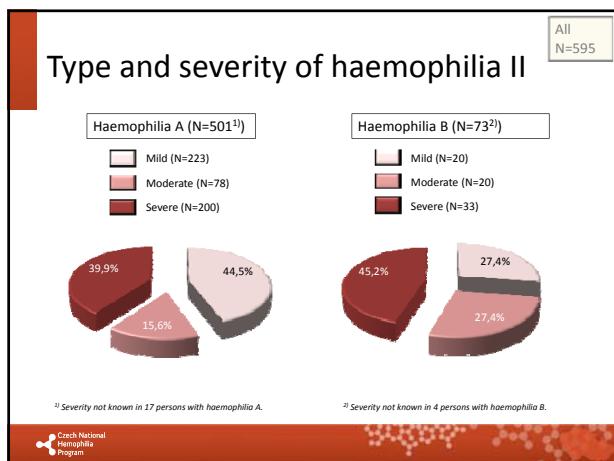


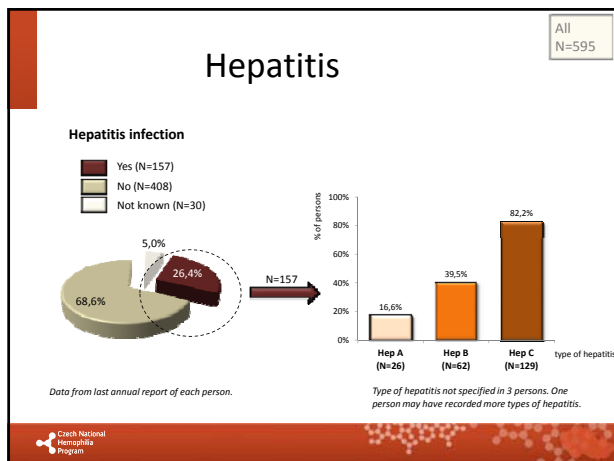
- Czech National Haemophilia Programme established
 - Multi disciplinary professional nation-wide initiative
 - All 4CCC + 7HTC involved
 - Regular accreditation of centres
 - Standards&Guidelines formulation
 - Promoting "European principles of haemophilia care"
 - Cooperation with health-care payers 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...

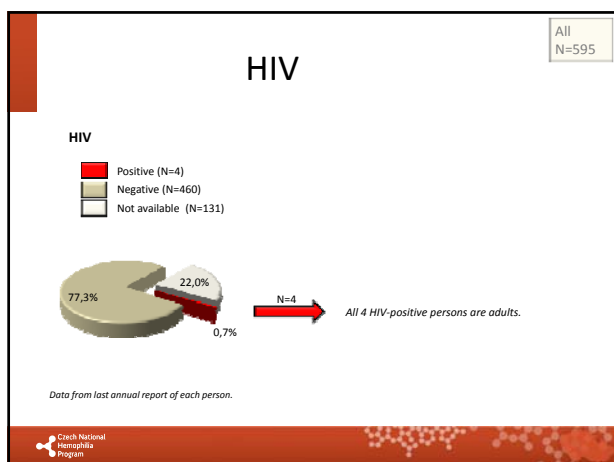
Czech National Hemophilia Program

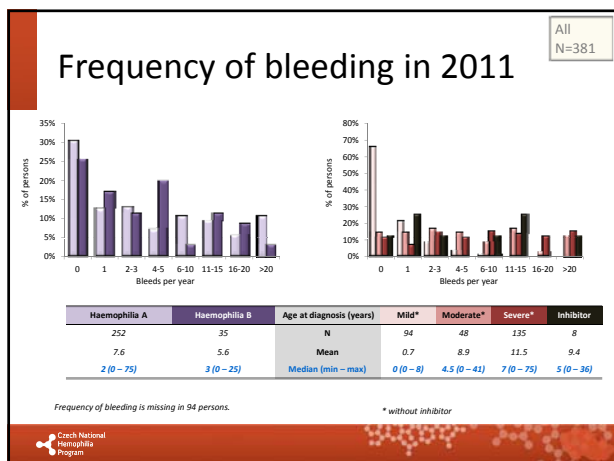
2011 CNHP REGISTRY DATA (ON REPRESENTATIVE POPULATION SAMPLE)

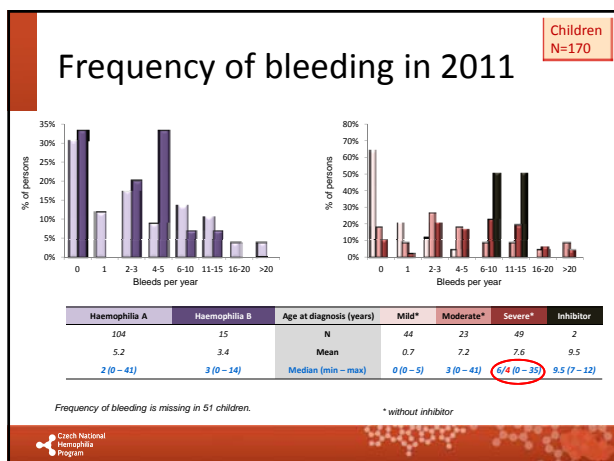


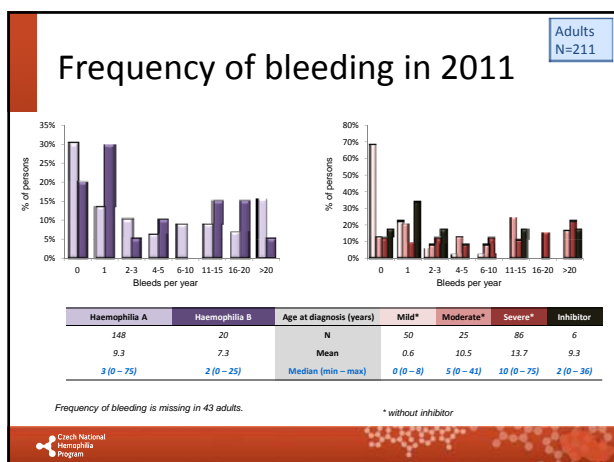


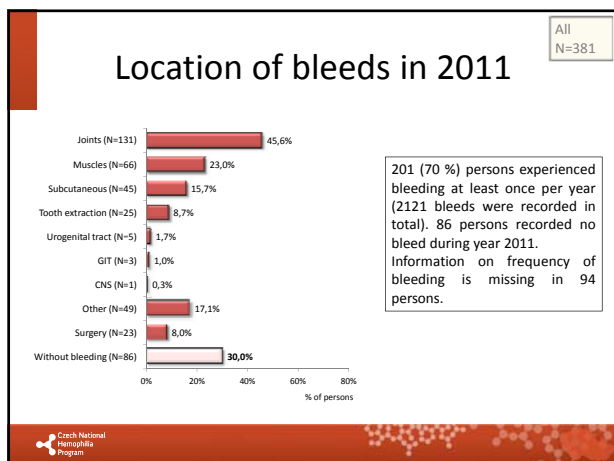


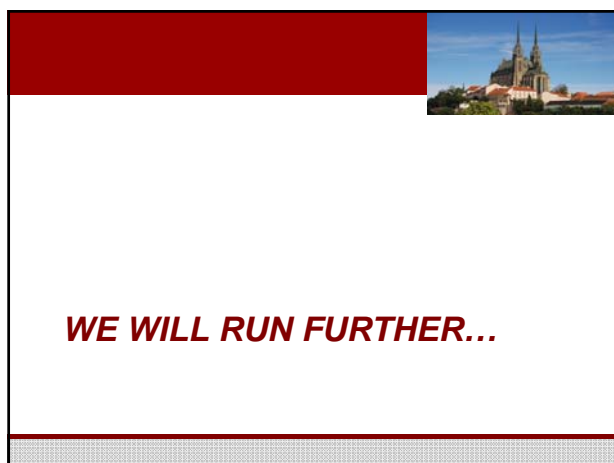












What are our "Czechallenges"



- Increase the factor consumption
 - Around 6IU/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
