CML HORIZONS
HYBRID MEETING 2022

CML ADVOCACY - LEARN, SHARE, GROW
20TH INTERNATIONAL CONFERENCE FOR ORGANISATIONS REPRESENTING PATIENTS WITH CML

CML ADVOCATES NETWORK

Marrakesh
Morocco
28-30 Oct 2022

HTA - THE CZECH PATIENT EXPERIENCE IN BLOOD CANCER

Jana Pelouchová
Diagnóza leukemie

EXCURSION TO HISTORY

“Categorisation Committee” at the Health Ministry - roundtable of stakeholders
(medical societies, pharmacist societies, payers, drug regulatory body, patient advocate)

- criticised by the EC for lack of transparency
- dismissed in 2007

B U T

- negotiations end 2006/2007 resulted in speedy reimbursement of Sprycel 04/2007

POLICY FOR HTA ASSESSMENT

Standard Reimbursement (2 options)

Option No. 1 - SIMPLIFIED APPLICATION (without CEA and BIA)

Option No. 2 - COMPLETE APPLICATION

- Clinical part, CEA (CUA) and BIA mandatory
- Clinical data must support reimbursement limitation (population)
- Local Epi data are mandatory, data from registry are welcomed
- Comparators: all relevant in required patient population / subpopulation
- Pre-defined threshold for ICER and Budget Impact must be kept
- New therapy must be cost effective vs. each comparator

POLICY FOR HTA ASSESSMENT - continued

Temporary Reimbursement

for Highly Innovative Medicinal Product (5 years)

- for innovative product with high efficacy

Temporary reimbursement for 5 years, then standard reimbursement (if not approved treatment of ongoing patient for free)

PATIENT INPUT

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Structured information by the patient organisation on the benefit of the new therapy:

- overview of the disease burden to the respective patient population/subpopulation
- psychological impact of the condition
- impact on self-sufficiency, limitations in everyday activities
- aspects of current therapeutical options
- patients' position on the benefit of new technologies
- patients' view on the administration
- concrete examples from patient surveys (quotes)

IMPORTANCE OF PATIENT SURVEYS

- generating robust data
- supportive information for local patient advocacy
- importance of patient networks vs. limited resources of local small organisations

OPTION FOR ASSESSMENT OF RARE DISEASE TREATMENTS

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New Legislation No. 48 in force from 1. 1. 2022

- newly established Advisory Board of the Minister of Health, for rare disease drug assessment. (formed by Ministry representatives, medical societies, payers and **patient organisations)**
- thanks to the **definition of patient organisation** within the Czech legislation No. 48
- Advisory Board of the Health Minister issues an obligatory Position Statement, for Drug Regulatory Body to elaborate into Final Decision

PATIENT ORGANISATIONS INVOLVED IN THE ASSESSMENT PROCESS

Part one: information on the disease + therapy

- *a)* organisation supporting patients with the particular disease
  (if non existing, the Czech Association of Rare Diseases takes care of)

providing Questionnaire on the burden of disease, current therapy and potential benefits of the new therapy

- social issues to be taken into consideration (self-sufficiency, ability to work, impact on caregivers)

Part two: decision-making process

- *b)* patient organisations who have expressed their willingness to actively participate in the Advisory Board (conflict of interest: from DIFFERENT disease area)

EXISTING GAPS

- membership at the **Innovative Therapies Working Group of the Health Ministry**
- group identifies some existing gaps

  (originally drug included in the Orphan Registry, not anymore)
  - permanent approval (pharmacoeconomics)
  - temporary approval for Highly innovative products (3+2 years - no renewal)

ULTIMATE OPTION - § No. 16

Option for access to drugs as emergency intervention, the one and only effective treatment:

- Patient Council at the Health Insurance Agency
- Regular meeting per 3 months
- Patient advocates presenting issues relevant to particular patient population / individual patient
- legislation NOT intended for treatment optimisation
- importance of supporting data

Health Insurance Agency from the Prague Tower

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