

IHI Call Days | CALL 9



● A Helping Hand for People with no Treatment

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- A Helping Hand for People with no Treatment - Benchmarking Compassionate Use Programmes



Challenges and objectives

- Improving access to treatments / pharmaceuticals
 - EU patients are discriminated vis-à-vis compassionate use programmes (CUP) / early access schemes (EAS):
 - depending on Member State of residence, access to potentially life-saving treatments can differ by months or years
 - Currently limited information for industry to learn from CUPs, limited use of CHMP opinion
- Beneficiaries: patients with life-threatening / severely debilitating conditions who have exhausted all treatment options
 - Investigational products may be the only treatment option remaining for these patients who cannot wait until their eventual approval, however the practice to enable access for patients varies and makes it difficult for Patients/HCPs
- IHI specific objective: T2 (SO2)
 - integrate fragmented health research and innovation efforts bringing together **health industry sectors and other stakeholders**, focussing on **unmet public health needs**, to enable the development of tools, data, **platforms**, technologies and **processes for improved prediction, prevention, interception, diagnosis, treatment and management of diseases, meeting the needs of end-users**

To demonstrate the public health utility of compassionate use programmes

- Prospective follow-up of compassionate use programmes / early access schemes with collection of data on their impact:
 - For patients: health outcomes, psychological relief
 - For clinicians: earlier experience with innovative products
 - For regulatory authorities: larger safety database, different population than in clinical trials
 - For payers: cost-benefit analysis, early RWD
 - For developers: faster uptake

A perfect project for a public-private partnership

- Private partners: pharmaceutical companies planning to initiate compassionate use programmes / early access schemes from the beginning of the project
 - Collecting data on difficulties initiating/conducting such programmes in EU Member States
 - Comparison of practices / approaches between companies
 - IT developers: creation of information resources/registries
- Public partners:
 - Patient organisations: defining patients' needs, characteristics of the programmes
 - Academy: measuring impact (outcomes, including psychological benefits, economic aspects...)

Outcomes and Impact

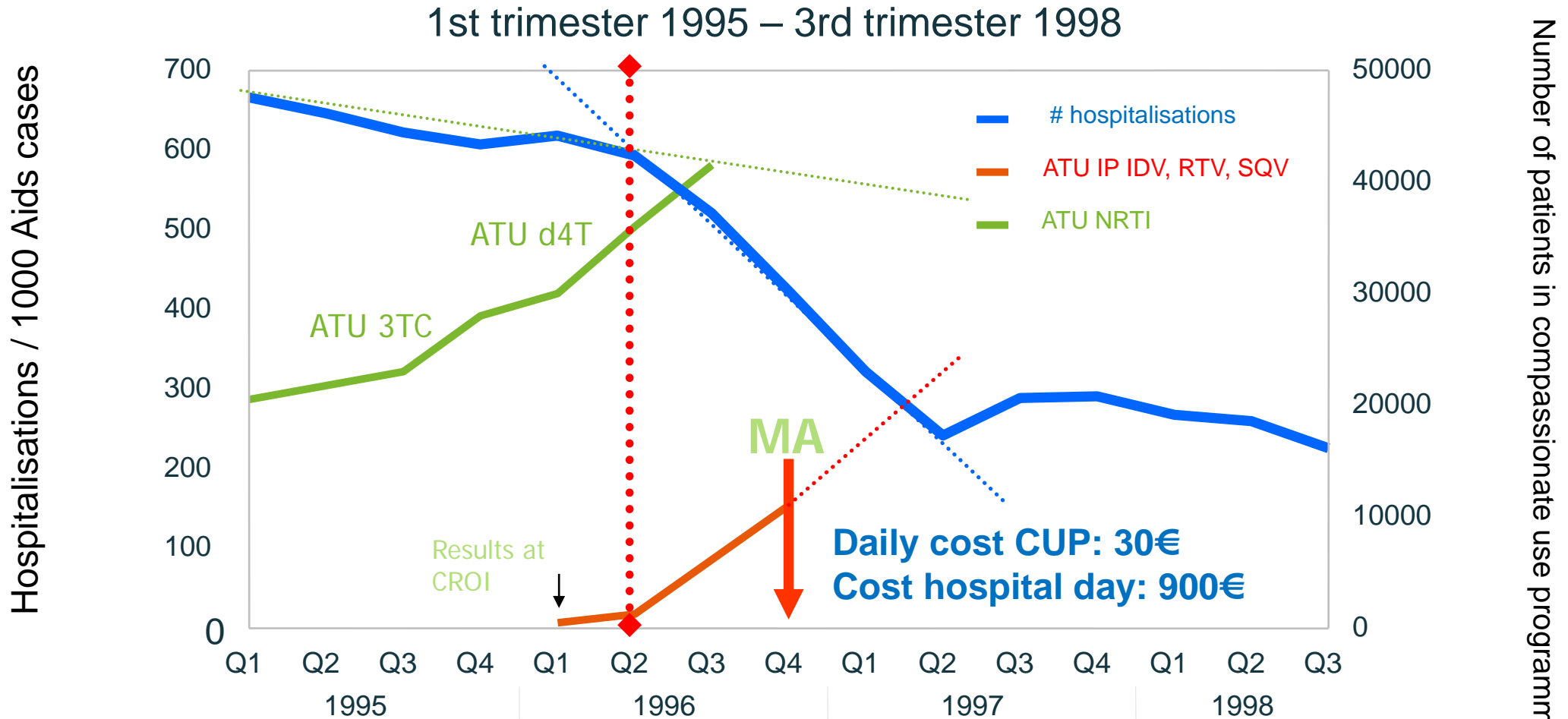
- Benchmark of effective solutions and effective measures that ensure early access to innovative medicines for patients in greatest need of new options (evidence-based policy)
- Sharing results with national regulatory agencies / authorities and empowering patient organisations and learned societies on the importance for healthcare systems to better harmonise compassionate use programmes
- Faster uptake of pharmaceuticals can represent a strong incentive for developers of medicines and increase the attractiveness of the European market. Earlier RWD can facilitate the decision-making both for regulators and for HTA
- More equity in accessing CUP / EA schemes in all EU MS, wherever patients live, and earlier realisation of public health benefits

Expertise and resources

- We have:
 - Patient organisations and Community Advisory Boards
 - Pharmaceutical companies that advocate for further streamlining of CU in the EU with EFPIA, EuropaBio
 - Partners specialised in the transferability of outcomes and economic impact in Eastern/Central European States
 - HTA bodies who conducted policy sandboxes on new payment models and early access schemes
 - Research organisations experienced in solutions for compassionate use programmes for single-administration medicines / ATMPs
- We are looking for:
 - Academic teams
 - Psychologic impact of health interventions
 - Measuring health outcomes of compassionate use programmes
 - Services
 - Development of IT tools to inform patients and clinicians of existing programmes

Cost-benefit analysis of a compassionate use programme

Hospitalisation rates for 1000 AIDS patients, France 1995-98



Sources: Hospitalisation rates DMI2 - Direction des Hôpitaux - BEH n°44/96
 Abbott Laboratories, Merck Sharp & Dohme, Glaxo Wellcome, Bristol Myers Squibb, Roche

