# eunethta magazine

WINTER 2017

eunethta

## HTA REPORTS

## IMPACT
CROATIA/ITALY

## INTERVIEW
AARNOUT/AIM

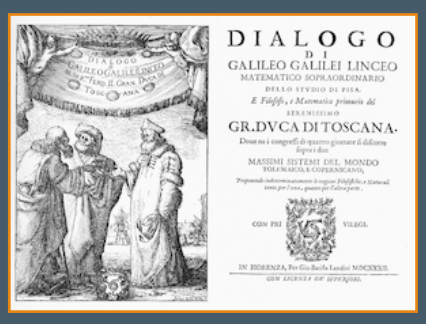
## TREATMENT
A PATIENT'S VIEWPOINT

## PARTNER PROFILES
PORTUGAL

## EMA
JOINT WORK PLAN 2017-2020

# IN THIS ISSUE



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12

13

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EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT


**EUnetHTA Magazine**





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*cover: Bill Branson*


# ON THE HORIZON

In this issue, we reflect on the state and progression of patient care and health care providers. In Three Questions, expanded to Four Questions as a gift for the holidays, we have Menno Aarnout, Executive Director of the International Association of Mutual Benefit Societies. Our stakeholder focus continues in a discussion with Dr. Cees Smits, who talks about past patient care and possible future outcomes for HTA. We also celebrate twenty-five and ten-year anniversaries from our partners in Portugal, INFARMED and ACSS IP.

EUnetHTA reached a landmark this quarter with the publication of the first joint JA3 pharma assessments, **PTJA02 on "Regorafenib (Stivarga©) indicated as monotherapy for the treatment of adult patients with hepatocellular carcinoma (HCC) who have been previously treated with sorafenib"**, and the second joint pharma assessment **PTJA01 on "Midostaurin (Rydapt©) with standard chemotherapy in FLT3 positive Acute Myeloid Leukaemia".**




"Disclaimer" The contents of this magazine arise from EUnetHTA JA 3 which has received funding from the EU, in the framework of the Health Programme. Sole responsibility for its contents lies with the author(s) and neither the EUnetHTA Coordinator nor the Executive Agency for Health and Consumers is responsible for any use that maybe made of the information contained therein.

# EUnetHTA Jointly-Produced HTA Reports

PTJA01 on "Midostaurin (Rydapt©) with standard chemotherapy in FLT3 positive acute myeloid leukaemia (AML)"

PTJA02 on “Regorafenib (Stivarga©) indicated as monotherapy for the treatment of adult patients with hepatocellular carcinoma (HCC) who have been previously treated with sorafenib”

OTCA01 on "Wearable cardioverter-defibrillator (WCD) therapy in primary and secondary prevention of sudden cardiac arrest in patients at risk"

OTCA02 on "Antibacterial-coated Sutures Versus Non-Antibacterial-Coated Sutures for the Prevention of Abdominal, Superficial and Deep Incisional, Surgical Site Infection (SSI)"

OTCA05 on “Repetitive transcranial magnetic stimulation for treatment-resistant major depression”

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NATIONAL IMPLEMENTATION AND IMPACT

*Written by Zoe Garrett, NICE*
*zoe.garrett@nice.org.uk*

**These articles are part of the work of WP7 National implementation and Impact**

The Agency for Quality and Accreditation in Health Care and Social Welfare (AAZ) has three strands of work: quality, accreditation and health technology assessment (HTA). The HTA department produces national HTA for a wide range of health technologies to support decision making by the Croatian Health Insurance Fund (CHIF), the Ministry of Health and hospital managers.

HTA in Croatia is not currently mandatory. Therefore, decision makers do not have to use HTA or request HTA from AAZ. AAZ is requested to provide HTA in situations where the decision makers require further information to inform their decision.

The HTA department produces approximately eight multiple technology assessments per year, including involvement as authors and co-authors on EUnetHTA reports. Reports can include relative effectiveness assessment only or full comprehensive assessment (but without primary full economic analysis).

# USE OF EUnetHTA ASSESSMENTS AT THE AGENCY FOR QUALITY AND ACCREDITATION IN HEALTH CARE AND SOCIAL WELFARE (AAZ)

## Working Practices

Requests for assessment come to AAZ from the Ministry of Health, CHIF or hospital managers. AAZ have a topic proposal form which institutions submitting requests may use to describe the topic and research question that they want addressed.

The HTAs are produced by AAZ staff and are approximately 70 pages long. The reports include information about the condition, the technology, clinical evidence, cost and a summary of published cost effectiveness evidence. For non-pharmaceutical health technologies relevant information about organisational, legal and ethical issues will also be added to the report. The type of information added is based on a shorter adapted version of the assessment elements in the EUnetHTA HTA Core Model. Where possible the assessment will make use of existing HTA assessment either created by EUnetHTA or another national agency.

AAZ include recommendations in their report. These include information about the use of a technology and also how a technology should be used. Once AAZ have delivered a report they are not involved in the decision making.

For pharmaceutical assessments AAZ must deliver their report one month after the request, which is possible only if appropriate recently published (by EUnetHTA or another national agency) report is available for adaptation. For non-pharmaceutical health technologies time frames are not defined by law and can be negotiated.

## Use of EUnetHTA assessments

AAZ have made use of the EUnetHTA assessments of abdominal aorta aneurysm screening, canagliflozinfor for the treatment of type II diabetes, ramucirumab for the treatment of advanced gastric or gastro-oesophageal junction adenocarcinoma and antibacterial-coated sutures for the prevention of superficial and deep incisional surgical site infection (SSI).

When using an existing HTA assessment created by either EUnetHTA or another national agency, AAZ will make the following adaptations to the assessment for use in the Croatian context:

·Update literature searches to identify and include any new clinical evidence and ongoing studies;

·Add epidemiological information such as patient numbers, morbidity and mortality in Croatia;

·Add information about the technologies available in Croatia;

·Add information about costs of relevant technologies in Croatia;

·Add any additional information required such as a summary of cost effectiveness evidence and organisational or legal issues.

AAZ must translate any information taken from an existing report into Croatian because the language of assessment must legally be Croatian.

For EUnetHTA assessments regardless of whether AAZ have been involved, AAZ prepares a summary in Croatian that will be published on the AAZ website with links to the full EUnetHTA assessment. These documents are not used for decision making, but if a decision maker subsequently requests the topic as an assessment from AAZ, the report is updated with the additional information required for the Croatian decision making context including recommendations.

Drivers that support the use of EUnetHTA assessments include:

·Having a methods guide that includes use of EUnetHTA

**continued on page 7**


# NATIONAL IMPLEMENTATION AND IMPACT (CONT.)

*Written by Zoe Garrett, NICE*
*zoe.garrett@nice.org.uk*

**These articles are part of the work of WP7 National implementation and Impact**

In Italy, there is currently no national-level decision making about pricing and reimbursement of non-pharmaceutical health technologies. Decisions about purchasing are made either at a regional level or at a hospital level. The Ministry of Health until recently has commissioned the Agenzia nazionale per i servizi sanitari regionali (AGENAS) to carry out national level HTA and horizon scanning in collaboration with regional HTA agencies, to provide evidence to support regional and local decision making about resource allocation. However, there was no formal process for taking HTA information into consideration during decision making processes and the extent to which HTA has informed decision making varies between regions and hospitals. In order to promote the cooperation among regions, in March 2015, a new national HTA system has been created. A steering Committee “Cabina di Regia” including representation from the MoH, national agencies and regions has been established to manage the National programme for HTA. AGENAS is in charge of coordinating the national network that manages assessment activities and supports capacity building of all the regions. Furthermore, in January 2017, the National Centre for HTA was established within the National Institute of Public Health (ISS). It will support the Steering Committee in order to improve quality, standards and value for money and to integrate HTA principles and methodologies into practice for planning public health services.

## COLLABORATIVE HTA OF NON-PHARMACEUTICAL HEALTH TECHNOLOGIES IN ITALY

**Developments in the procedures for the assessment of non-pharmaceutical health technologies**

The National network managed by the Steering Committee has taken the place of the Italian Network for HTA (RIHTA), a voluntary network, established in 2009, including AGENAS and representatives of 13 of the 21 regions. The aim of the network was to reduce duplication in assessment and support training and capacity building.

Topics for assessment were sent to the MoH from a public notification system and were prioritised by a Committee including the MoH, AGENAS and representatives of the regions. Work allocation was managed by AGENAS depending on agencies and regions capacity and interest in the topic.

Work on each assessment was divided between two-four agencies. Agencies producing assessments followed a manual produced by the network including procedures and methods to be used and set out the structure of the assessment. Reports were completed using an adapted version of the HTA CORE model and EUnetHTA tools to support assessment. The reports included all areas of HTA.

The assessments completed through the RIHTA network contained recommendations to inform national decision making, regional healthcare directorates, procurement agencies and local healthcare trusts and hospitals. The reports were subject to public consultation before being finalised and were published on the Ministry of Health and AGENAS' websites. The advice in them was not mandatory.

Building on the success of RIHTA network, as outlined above, a law was established in 2015 to support further cooperation in HTA production and use. This includes mention of the use of EUnetHTA tools and products.

The law establishes a national steering Committee “Cabina di Regia” that includes representation from the MoH (3pp), AIFA and AGENAS (2pp) and four regional representatives who will be overseeing interest of the regions. The Committee is tasked to define priorities for assessment in line with European guidelines (for example EUnetHTA guidelines), promote and coordinate the activities of assessment, validate the methodology for assessment, manage publication, dissemination and evaluation of the assessments, promote their use by the regions and health agencies so as to inform decisions on the adoption and introduction of medical devices and their disinvestment.

Proposals for topics will be received by AGENAS and prioritized by the Steering Committee of the Ministry of Health. Priority topics will be those that meet the prioritisation criteria and which come from groups of regions or the national committee for healthcare. Prioritisation criteria include the potential impact on the healthcare pathway, any ethical and social implications (particularly in regard to quality of life and

**continued on page 7**

# INTERVIEW: MENNO AARNOUT/AIM

## EXPANDING DIALOGUE BETWEEN HTA BODIES AND STAKEHOLDERS

Each quarter, EUnetHTA Magazine endeavours to ask three questions to a key stakeholder focusing on issues in the HTA world. For our winter edition and the holiday season, we asked Menno Aarnout, Executive Director of AIM, the International Association of Mutual Benefit Societies (Association Internationale de la Mutualité), four questions.

About/from AIM:

The International Association of Mutual Benefit Societies (AIM) is an international umbrella organisation of (national federations of) healthcare payers; mutuals and health insurance funds, with 64 members from 31 countries around Europe but also from Latin America, Africa and the Middle East. AIM members are all democratically governed, solidarity based, not-for-profit organisations providing health coverage to around 240 million people worldwide. AIM's objective is to defend and strengthen universal access to high-quality, affordable healthcare.

*AIM was created in 1950. In 1998, AIM moved its HQ from Geneva to Brussels. It might be said that the focus could be perceived as European. However, AIM is a European-based association representing organisations in Europe, Latin America, Africa and the Middle East.For payers, how can European HTA assist in having a much broader impact on the sustainability of healthcare, not just in Europe?*

AIM and its members think that it is highly important to assess the effectiveness of health technologies, not only when a product arrives newly to the market, but also when technologies have been on the market for a while already. The efforts within EUnetHTA don't have as their single aim to reduce duplication of efforts among EU member states, but also to further increase the quality of the assessment, contributing to effectiveness and cost-effectiveness of healthcare systems. Methodological improvements can also be a benefit for organisations that carry out HTAs outside the European Union and for healthcare systems worldwide.

*EUnetHTA is a network of 81 organisations in 29 countries usually organized at the national level.What is your perspective on how EUnetHTA and its members can support the implementation of the EU Directive (2011/24/EU) on patients' rights in cross-border healthcare?*

Increasing cross-border mobility of EU citizens makes collaboration in the field of healthcare between countries highly relevant. The Directive facilitates this collaboration and provides a framework for ensuring patients having excess to good quality care, be it in their own country of elsewhere. One of the aims of the Directive is to ensure that EU countries work closer together in the interest of patients. Support of collaboration in the field of HTA is an important element of the directive. AIM considers it highly important that the collaboration is not limited to collaboration between HTA agencies alone. It is interesting to improve the quality of the assessment, but that should be done together with the end-users of those assessments. Many stakeholders, including the members of AIM, rely on the work of the HTA agencies. It is important that, also at EU level, HTA agencies and stakeholders work together. Until today EU collaboration in the field of HTA has been something mainly for the HTA agencies, without seriously trying to involve other stakeholders in the process.

*The World Health Organisation defines HTA as the systematic evaluation of properties, effects, and/or impacts of health technology. It is a multidisciplinary process to evaluate the social, economic, organizational and ethical issues of a health intervention or health technology. The main purpose of conducting an*

***Menno Aarnout***

Twiiter feed: AIM, The International Association of non-for-profit healthcare payers represents health mutuals and health insurance funds in Europe and in the world.

***https://www.aim-mutual.org/***

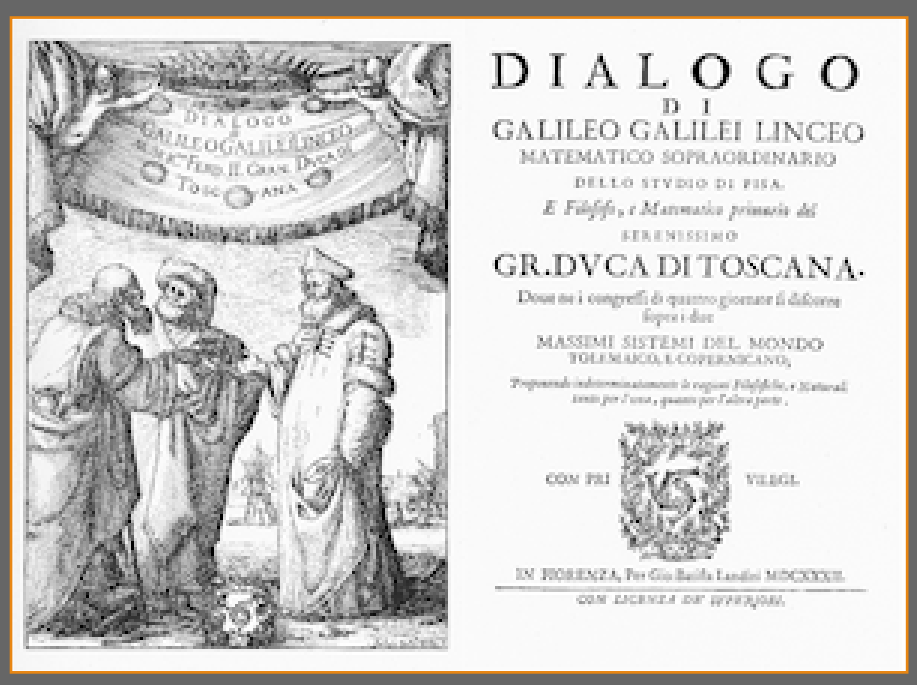
@AIM_Healthcare

THREE QUESTIONS

# INTERVIEW: MENNO AARNOUT (CONT.)

*assessment is to inform policy decision making.In this context, and speaking directly as a primary voice for payers, how should HTA be directed in informing policy decision making?*

Doing a health technology assessment is a good thing, but the outcome of the assessment should be used, by health professionals, by patients, by the industry, by decision makers and by healthcare payers. It is thus important that HTA agencies don't work in isolation. They should reach out to the end-users of their assessments to discuss whether the assessments are useful, and to discuss which technologies should get priority when the assessment agenda is being prepared. This dialogue between HTA bodies and relevant stakeholders is at EU not yet well-developed.




*What options do you see to increase the involvement of payers in EUnetHTA activities?*

I would suggest that healthcare payers and HTA bodies sit together and see where we can help each other. AIM has invited EUnetHTA at several occasions to see how healthcare payers can be involved in the work of the network. We see different fields where we can collaborate. In the first place we see possibilities when it comes to post marketing evidence generation, but AIM members would also like to be involved in discussions about which technologies are most in need of an EU HTA assessment.

*Since 2014, Menno Aarnout has worked for AIM. Before that he held different positions in the Dutch Ministry of Health and the European Commission, where he specifically contributed to the EC agenda on Health Technology Assessment. In 2006-2007, he was secretary of the working group on relative effectiveness, in preparation for the Pharmaceutical Forum. Within DG SANCO he was also a member of the team that prepared the 2011/24/EU Directive on the application of patients' rights in cross-border healthcare. That directive contains article 15, which states, "The Union shall support and facilitate cooperation among national authorities or bodies responsible for health technology assessment".*

# IMPACT: CROATIA (CONT.)

**(continued from page 7)** assessments and use of other national assessments as a basis for carrying out HTA;

· Identifying and using existing HTA reports from EUnetHTA and other jurisdictions is built into their procedures and ways of working;

· Being able to negotiate timelines for non-pharmaceutical health technologies.

In addition, pharmaceutical launch is often delayed in Croatia which means that HTA reports from EUnetHTA or other jurisdictions are often available for use.

**Grateful acknowledgement to AAZ**: Mirjana Huić (Agencija za kvalitetu i akreditaciju u zdravstvu i sociijalnoj skrbi/AAZ)

# IMPACT: ITALY (CONT.)

**(continued from page 7)** sustainability of care), potential organisational impact, potential economic or financial impact, technical relevance of the technology in the healthcare pathway, uncertainty in effectiveness and epidemiological significance.

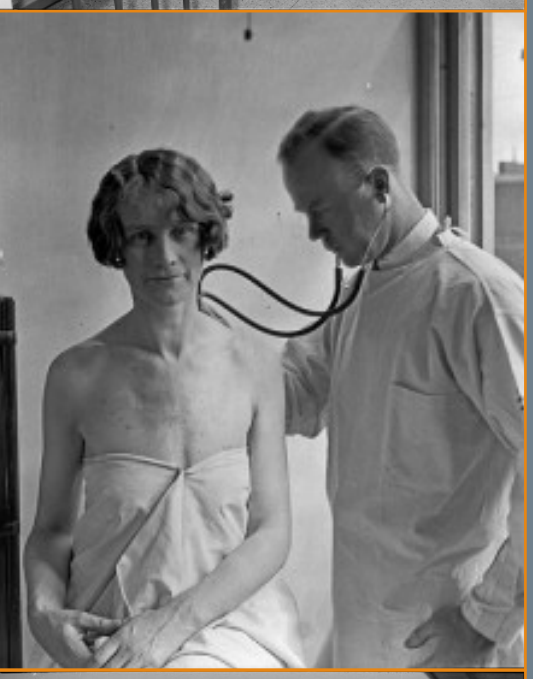
Selected topics will then be allocated for assessment to regional agencies or other public or private HTA units. Assessments will be completed collaboratively and the network will have a common approach and share methods and objectives. Coordinating activities will be performed by Agenas.The reports will be appraised by the Steering Committee and will produce recommendations that will be mandatory for the system. There will be input into the assessment from all regions (e.g. through consultation) but the decision will be taken by consensus at the Steering Committee. The recommendations from the reports will feed into the central procurement Committee and the Commissione Nazionale LEA (the Committee for Healthcare) to support definition of the minimum basket of health care.

A strategic paper has now been released to be adopted by the national Government and Regions. The next step will be to define the initial reports, that will act to pilot the process. Following the pilot an annual work plan will be developed which will include both assessments and also training, capacity and methodological requirements.

**Grateful acknowledgement to ITALY**:

Maria Grazia Leone (Ministero Della Salute (DGFDM IT)), Pietro Calamea (Ministero Della Salute (DGFDM IT), Marina Cerbo (Agenzia Nazionale Per I Servizi Sanitari Regionali (Agenas)), Luciana Ballini (Agenzia sanitaria e sociale regionale - regione Emilia-Romagna (ASSR RER)), Marco Marchetti (The HTA Unit in the Agostino Gemelli University teaching hospital (UCSC - Gemelli))



# THE EVOLUTION OF TREATMENT

## A PATIENT'S VIEWPOINT: CONTRIBUTION FROM DR. CEES SMIT

At the September 2017 EUnetHTA Forum in Amsterdam, the multi-stakeholder panel discussion had an engaging presentation from Dr. Cees Smit. Dr. Smit has seen and experienced the history of HTA. He also represents that a patient's viewpoint on HTA should and can be a valued part of HTA.

Dr. Smit said, "*I was born in 1951 with a severe form of haemophilia, a rare disease for which, at that time, there was no real treatment. My parents were told that my life expectancy would be rather short: around 20-30 years. In my youth, I stayed on average almost half a year annually in the hospital because of spontaneous bleeding in my joints and muscles.*

*As there were no treatment options, my parents were advised many kinds of what would we now call "alternative therapies". In my sixth year, I was treated with DES (di-ethyl stilbestrol) with the idea that while it would stop the growth process, it would also stop internal bleeding. That didn't work at all but it did cause short stature. Later, we heard a lot of other side-effects of DES, especially in pregnant woman and their children."*

Dr. Smit continued, "*A treatment for haemophilia became available in the mid 1960s. By isolating the clotting factor as a bloodplasma product, this treatment certainly changed my life. I had almost no hospitalization anymore. I could perform intravenous injections at home, at work and even during travel. The quality of my life changed dramatically. I often call haemophilia a pre-orphan disease. There was already a treatment in place before the US and EU Orphan Drug Acts.*

*In the 80s, haemophiliacs experienced many problems with viral infections in bloodplasma products, especially when they were prepared by paid US plasmapheresis donors. Luckily enough, I did not became immediately ill, but did after 10 years. At the time, there was already the first drug for HIV, AZT, and later other effective treatments became available. Now my life has been prolonged by effective new innovative treatments, for hemophilia, HIV and HCV.*

*The consequence of this is that I am now a complex patient with comorbidity because of these unavoidable (but avoidable) and unexpected side-effects of all these different treatment regimes.*

*Another consequence is that although haemophilia treatment was a rather cheap treatment 50 years ago, it is now somewhat costly. This is because of the viral inactivation steps needed in the production process of bloodplasma products or biological versions of clotting factors. The problem with biologicals is that they may probably cause more antibody formation, which makes treatment even more expensive.*

*In the Netherlands, we already started to evaluate the effectiveness of modern haemophilia treatment in 1971 in terms of medical and social outcome measures. We also published about these quite early (British Medical Journal: Physical condition, longevity, and social performance of Dutch haemophiliacs, 1972-85. 28/1/89). The medical outcome is almost an n = 1 experiment: it works or does not work. One treatment is enough to show its effectiveness.*

*However, haemophilia treatment is not the problem. The real problem is the needed blood transfusion infrastructure in Europe to guarantee the supply of high-quality plasmaproducts at a low cost. Almost all not-for-profit transfusion services in the European market disappeared or were sold to companies in the US or China. This is highly undesirable as blood and plasma products are strategic goods for Europe. They should not be endangered by growing demand outside the continent, or political unrest."*

This brings us to the concept that European HTA, and HTA in general, should not only continue to help prevent financing technologies with limited or no-added value, but should also possibly assess treatments that may be limited in scope but have effective cost savings. The two ideas are not mutually exclusive. While EUnetHTA

remains neutral, our role continues to be providing assistance in the development of reliable, timely, transparent and transferable information to contribute to HTAs in European countries. This involves facilitating efficient use of resources available for HTA, creating a sustainable system of HTA knowledge sharing and promoting good practice in HTA methods and processes.

Patients and their viewpoints are an important part of the HTA process. Dr. Smit gave an example of a possible treatment for haemophiliacs. Dr. Smit said, *“For the future, the haemophilia community, as well as HTA scientists and payers, should be proactive to find better solutions. Take gene therapy in haemophilia. Although initially available at a high cost, data now available shows a substantial savings as opposed to yearly treatment costs that national payer systems make. For instance, for the price of 750.000 euro for an outlay on an initial gene therapy, which may seem high, these costs can be saved in six years for a patient with severe haemophilia. It also may be much more cost-effective in patients with antibodies. A new financing model (a lease construction) is highly needed.”*

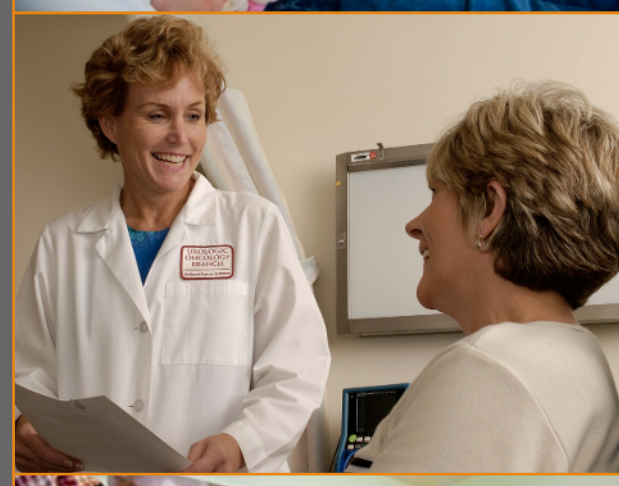
The US' National Public Radio echoed this viewpoint on November 29, 2017 in *Gene Therapy Shows Promise for A Growing List of Diseases* (goo.gl/13yS19). The price tag remains the question but patients "helped by these treatments so far seem delighted". This hypothetical savings model might encourage a rational way forward.

HTA continues to be a dynamic and fluid field where payer, patients and health care provider viewpoints should be considered. Frank discussion on HTA works, but only if everyone’s viewpoint is taken into consideration whenever possible. As Dr. Smit pointed out at the EUnetHTA Forum, there are multiple EU Patient Advocates well-trained by now to accept the challenge of an HTA dialogue with all relevant stakeholders.

**Cees Smit** studied business economics at the Free University in Amsterdam.

From 1978 till now, he has been a member of the research project ‘Haemophilia in The Netherlands’ at the Leiden University Medical Centre (LUMC). From 1987 till 1998, he was co-ordinator of the Netherlands Haemophilia Society, after which he worked in the mental health area. In January 2003, he received an honorary doctorate from the College of Deans of the University of Amsterdam in recognition of his work on patient participation, haemophilia and medical biotechnology. At ZonMw (The Netherlands Organisation for Health Research and Development), he is on the Commission for Translational Gene Therapy and Stem Cell Therapy. He is also chair of the Policy Board of the Biobank of Radboud UMCN.

In recent years, he wrote several books on ageing with chronic diseases, like haemophilia and HIV. ‘Ageing with Haemophilia’ was published in English in 2007. The book ‘Ageing with HIV’ was published during the International Aids Conference in Vienna in 2010. In September 2015, together with Annemarie de Knecht – van Eekelen, he published the book ‘De macht van de patiënt, baas over je eigen ziekte’, which would translate in English as ‘The power of the patient, boss of your own disease’. One of the issues covered in this book is the increasing cooperation between patients, researchers and industry in the drug development process (Patient 3.0).



# PARTNER PROFILES: PORTUGAL

## INFARMED CELEBRATES 25 YEARS

**The Beginning**

After Portugal's ascension to the European Union (then the EEC), the first collective medicines law, the Medicines Statute, was introduced in 1991. This brought together different laws into a regulation framework, which until then were dispersed, allowing modernization of the entire medicines area in Portugal.

In addition to this statute, in 1993, two years before the creation of the European Medicines Agency (EMA), it was a priority to organize, together with all partners in the sector, a system of guaranteeing the quality, safety and efficacy of medicines. January 15, 1993 saw the creation of INFARMED, which completes, in January 2018, 25 years.

**25 years of consistent evolution**

Today, INFARMED, as Portuguese National Authority of Medicines and Health Products, is responsible for the regulation and supervision of medicines for human use and health products (medical devices and cosmetic products). INFARMED has a staff of about 350 highly qualified professionals in several areas, in particular the life sciences. In its activities, INFARMED also counts on the support of expert committees, gathering 300 people from academic, research and health institutions.

Throughout the years, in order to fulfil its mission and vision, INFARMED has developed a set of initiatives to play a fully-fledged role in European regulation, to upgrade its means and resources, and to promote efficiency and quality management.

The prestige of INFARMED and its participation in the scientific evaluation processes of EMA contribute to Portugal´s active role, both in the establishment of the European regulatory system for medicines, and in its development and consolidation. Another corollary effect of this participation noteworthy position occupied by the Portuguese regulator in the framework of the medicines evaluation procedures and in the coordination of committees and working groups of EMA.

Also, and since its creation, INFARMED has been responsible for assessing the effectiveness and the economics of medicines, by means of added therapeutic value and cost effectiveness tools, basing the reimbursement and pricing decisions in the Portuguese National Health Service.

In the context of the economic evaluation of medicines, Portugal witnessed a significant evolution since the end of 1998 with the adoption, by INFARMED, of methodologies used in carrying out studies and economic evaluation of medicines.

This led to the introduction of the term "cost-effectiveness" in the economic evaluation of medicines lexicon that was reflected in the management of new applications for reimbursement, in a large-scale re-evaluation of the reimbursements made in the year 2001 and in the introduction, in 2007, of a specific cost-effectiveness assessment for all medicines used in hospitals.

More recently, in 2015, INFARMED was responsible for the implementation of the National System of Health Technology Assessment (SiNATS), concerning the assessment of effectiveness not only of medicines, but also of medical devices and other technologies.

The main objectives of SiNATS are the maximization of health gains and the evolution of citizens quality of life, as well as ensuring the efficient use of public resources, reducing waste and inefficiencies, which contribute to the sustainability of the National Health Service.

The Portuguese HTA system intends to promote equitable access for citizens to the available health technologies, encourage and reward relevant health innovation, as well as the expand Portugal's involvement in the exercises for an integrated European HTA system (such as EUnetHTA).

Through measures that guarantee transparency, predictability, fairness in use and the attainment of health gains - that justify public funding, SiNATS therefore emerges with the aim of providing the NHS with a unique instrument that improves its performance by introducing best practices at European level with regard to the use of health technologies.

On the economic side of medicines, in recent years there has been a marked reduction in the burden on the NHS and the citizen, both through the evolution of the market for generic and biosimilar medicines and through price revisions or changes in economic evaluation and co-participation.

**Aiming towards the future**

Heading into the upcoming years, INFARMED is preparing its future (see PREPARING FOR THE FUTURE on next page), consistently working to meet increasingly demanding challenges at both the national and international levels. Such preparedness will allow INFARMED to maintain its standing as an agency of reference, recognized by its stakeholders, visible in the fulfillment of a public service with quality, rigor and transparency. This commitment is essential in the pursuit of a more efficient management of resources and ensuring all citizens fair, affordable and equal access to the medicines they need.

# INFARMED, I.P. MISSION

## REGULATE AND SUPERVISE

- ENSURE THE ASSESSMENT OF HUMAN MEDICINES IN TERMS OF QUALITY, SAFETY AND EFFICACY
- ENSURE HIGHER STANDARDS OF EXPERTISE IN PORTUGAL AND EUROPE

## ACCESS

- ENSURE THE COST-EFFECTIVENESS OF MEDICINES FOR HUMAN USE
- GUARANTEE EQUITABLE ACCESS TO QUALITY, EFFICIENT AND SAFE MEDICINES

## HEALTH TECHNOLOGY ASSESSMENT – HTA

- MANAGE THE NATIONAL SYSTEM OF HEALTH TECHNOLOGIES ASSESSMENT - SINATS
- CONTRIBUTE TO THE SUSTAINABILITY OF THE NATIONAL HEALTH SYSTEM WITH CHALLENGING INNOVATION

HUMAN MEDICINES

MEDICAL DEVICES

COSMETICS



- STRENGTHENING STRUCTURE AND ORGANISATION
- REINFORCE TECHNICAL AND SCIENTIFIC HUMAN RESOURCES
- PROMOTE CLINICAL RESEARCH ACTIVITIES AND METHODS
- RESPOND TO PATIENTS' NEEDS THROUGH REGULATORY AND HTA SYNERGIES

**Grateful acknowledgment to** Pedro Faleiro and Mjoao Morais

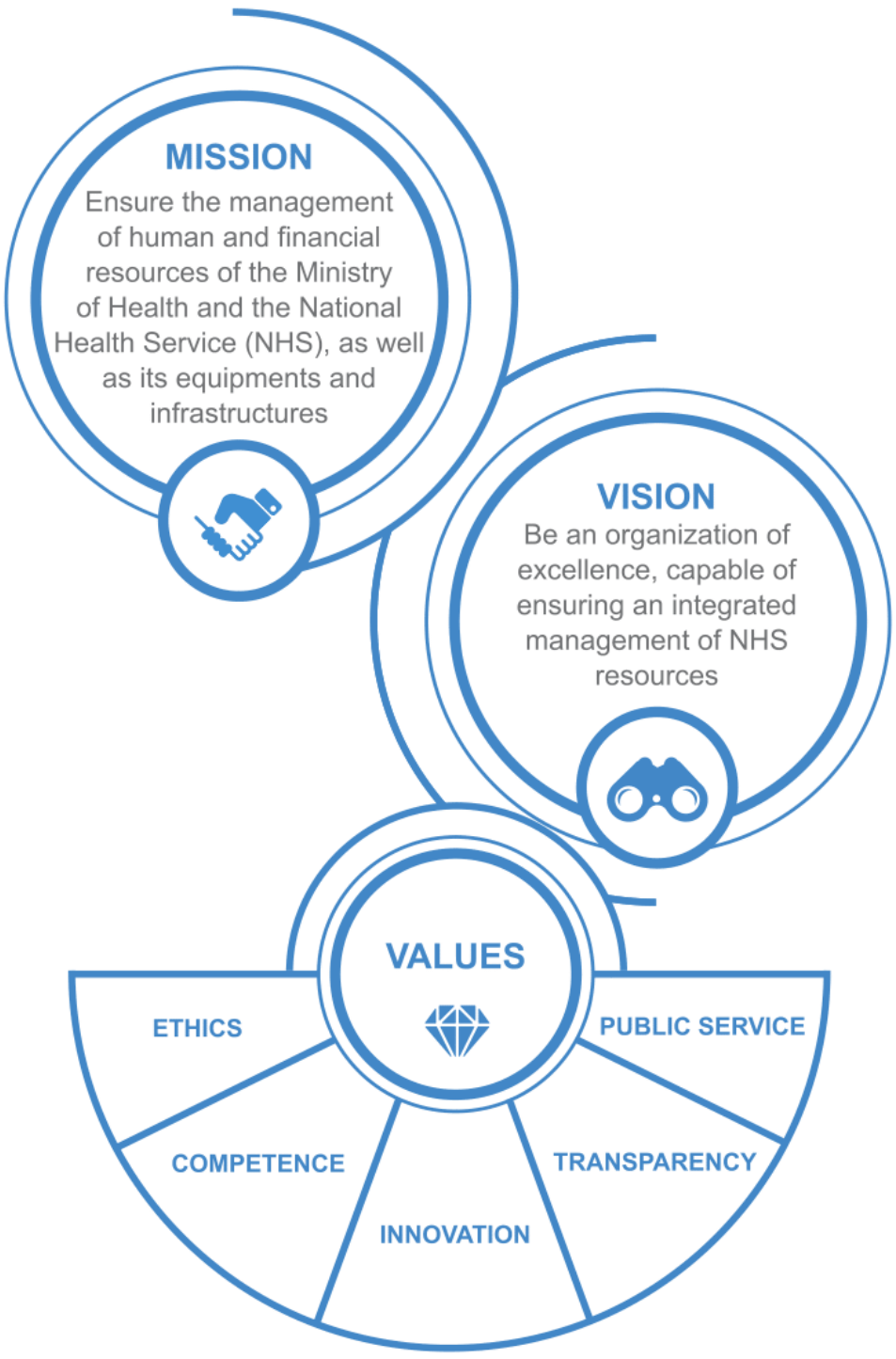
# PARTNER PROFILES: PORTUGAL

## ACSS IP 10-YEAR MILESTONE

ACSS ADMINISTRAÇÃO CENTRAL DO SISTEMA DE SAÚDE, IP

## OPTIMIZING RESOURCES, GENERATING EFFICIENCY

ACSS - Administração Central do Sistema de Saúde (Central Administration of the Health System) is a public institute established in 2007, embedded under indirect State administration, with administrative and financial autonomy, which fulfills the duties of the Ministry of Health, under its supervision and guardianship with jurisdiction over the national continental territory.




### ACTIONS

- Plan and coordinate the financial resources of the NHS
- Develop health workforce policies, including professional regulation
- Set financing models concerning the healthcare services contracting and monitor the implementation of contratcts with NHS hospitals
- Coordinates the management of health facilities and equipments in NHS, aiming at an integrated organization and rationalization of the hospitals network, primary health care, the Long-Term Care National Network, including mental health, and Palliative Care National Network
- Provide the NHS with the appropriate information and communication systems and purchasing rationalization mechanisms, through SPMS (Shared Services of the Ministry of Health)
- Coordinate and consolidate the assembly of information and statistics on production, health care performance, financial and human resources in the NHS
- Manage the NHS Centre of Control and Monitoring
- Coordinate the Access to Healthcare Integrated System
- Promote the shared management of resources in the NHS
- National contact point for cross border healthcare
- Prepare the implementation of the Public Health Initiatives Programme (EEA Grants)

### CHALLENGES

- Contribute for the efficiency and sustainability of the NHS
- Improve the governance of the health system
- Ensure equitable and consistent funding policies in line with the health policy
- Develop human capital and improve the performance of health-care professionals
- Increase transparency in the NHS

# EMA AND EUnetHTA FINALISE WORK PLAN FOR 2017-2020

## Medicines regulator and EUnetHTA continue to strengthen our collaboration

The European Medicines Agency (EMA) and the European Network for Health Technology Assessment (EUnetHTA) have published a joint work plan outlining key areas of collaboration for the next three years.

The EMA-EUnetHTA collaboration, which began in 2010, aims to harness synergies between regulatory evaluation and health technology assessment (HTA) along the lifecycle of a medicine whilst respecting their different remits. The overall goal is to improve the efficiency and quality of processes and ensure mutual understanding and dialogue on evidence needs. This facilitates improved access to medicines for patients in the European Union (EU).

“Regulators and HTA bodies have different responsibilities with regards to medicines. What unites us is a common goal of getting to the market more high-quality medicines that address unmet needs of millions of patients in the EU,” said EMA’s Executive Director, Professor Guido Rasi. “By working together, EMA and EUnetHTA help medicine developers to improve clinical research and become more efficient in generating the evidence each of us needs for good decision-making.”

“This work plan shows a broad commitment from the regulatory as well the HTA side to find clear synergy in our activities,” said EUnetHTA’s Director Wim Goettsch. “I believe that there is a common sentiment that these activities are essential to make our processes more transparent, efficient and timely for patients, producers and other key stakeholders.”

Some objectives of the new work plan include areas in which major progress has already been made, most notably:

- early dialogue / scientific advice: a new joint platform for parallel consultation was created in July 2017 to provide developers of medicines with simultaneous, coordinated regulatory and HTA advice on their development plans and facilitate alignment of data requirements;
- information exchange at market entry: the exchange of information on the outcome of the regulatory assessment at the time of marketing authorisation as part of EUnetHTA’s new framework for production of relative effectiveness assessments (REAs);
- post-authorisation data generation: post-licensing evidence generation tools, such as patient registries, are being optimised to serve data needs for various decision-makers.

In addition, EMA and EUnetHTA will further collaborate in a number of areas including:

- exploring how HTA bodies and regulators apply the concepts of unmet medical need and therapeutic innovation in view of possible synergies;
- understanding the conceptual similarities and differences between the significant benefit of orphan medicines versus their added therapeutic value.

These activities are directly related to the core activities of both organisations.

The three-year work plan is complementary to actions foreseen in EUnetHTA Joint Action 3, which runs until 2020.

Furthermore, the activities in this work plan will feed into the implementation of the areas for collaboration identified in the reflection paper from the HTA network on Synergies between regulatory and HTA issues on pharmaceuticals and will be developed in close cooperation with the European Commission.

**More on EMA-EUnetHTA collaboration**

The collaboration between EMA and EUnetHTA started in 2010. This collaboration began initially to address recommendations by the Pharmaceutical Forum, a high-level group of European policy makers, to improve the way data published by EU regulators as part of their benefit-risk assessment contribute to relative effectiveness assessments by HTA organisations. Furthermore, topics of mutual interest were identified and included in the work plan for 2012-2015, for which a report is available.

EMA and EUnetHTA hold regular bilateral meetings to progress the various actions. Their meeting reports are published on our respective websites.

Notes

- EUnetHTA is a network of organisations (from EU Member States, EEA and accession countries) and a large number of relevant regional agencies and not-for-profit organisations that produce or contribute to health technology assessment in Europe. EUnetHTA enables scientific cooperation between HTA bodies in Europe. It is co-funded by the Public Health Programme of the European Commission, DG Health and Consumers and performs the function of the scientific and technical cooperation of the HTA network established as per theDirective 2011/24/EU on the application of patients’ rights in cross-border healthcare.
- HTA bodies provide recommendations on the medicines that can be paid for or reimbursed by the healthcare system in a particular Member State.

THURSDAY 14 SEPTEMBER

# EUnetHTA Forum 2017

## 81 PARTNERS. 29 COUNTRIES. 300+ GUESTS.

## THE EVENT

This year's event was held in the heart of Amsterdam and brought together even more EUnetHTA partners, industry experts and national delegates than ever before. With an agenda ranging from discussions on the role of stakeholder involvement in horizon scanning, topic selection and prioritisation to additional data collection through different sources, the day offered a unique window into the advances Europe has made in the world of HTA over the past year.

## THE AGENDA

**12** TOPICS DISCUSSED

**29** SPEAKERS AND PANEL MEMBERS

**98%** HIGHLY RATED THE SELECTED PANELLISTS

**93%** HIGHLY RATED THE CHOSEN TOPICS

**84%** THOUGHT THE PROGRAMME OFFERED A GOOD BALANCE BETWEEN INFORMATION GATHERING AND NETWORKING OPPORTUNITIES



3 OUT OF 5 ATTENDEES USED THE EVENT TO MEET NEW POTENTIAL PARTNERS AND ESTABLISH CONTACTS WITH STAKEHOLDERS

TO FIND OUT MORE ABOUT THE EUNETHTA FORUM 2018 IN COLOGNE, VISIT EUNETHTA.EU OR EMAIL US AT EUNETHTA@ZINL.NL