European Commission

2014-2020

EU HEALTH PROGRAMME CONFERENCE

BRUSSELS
30 SEPTEMBER 2019

Consumers, Health, Agriculture and Food Executive Agency

Objective 4

'Facilitate access to better and safer healthcare for Union citizens'

Parallel Session II

European Commission

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Parallel Session II

European Commission

Chair: **Anna-Eva Ampelas,** **DG SANTE, European Commission**

Co-chair: **Georgios Margetidis,** **Chafea**

- European Reference Networks for Rare Diseases: **Irene Mathijssen**, Erasmus University Medical Center, the Netherlands
- Rare diseases: **Yann Le Cam**, European Organisation for Rare Diseases (EURORDIS)
- Substances of Human Origin: **Paola Di Ciaccio**, National Institute of Health, Italy
- Antimicrobial resistance: **Marie-Cécile Ploy**, INSERM, France
- European Pharmacopeia: **Michael Wierer**, EDQM, Council of Europe

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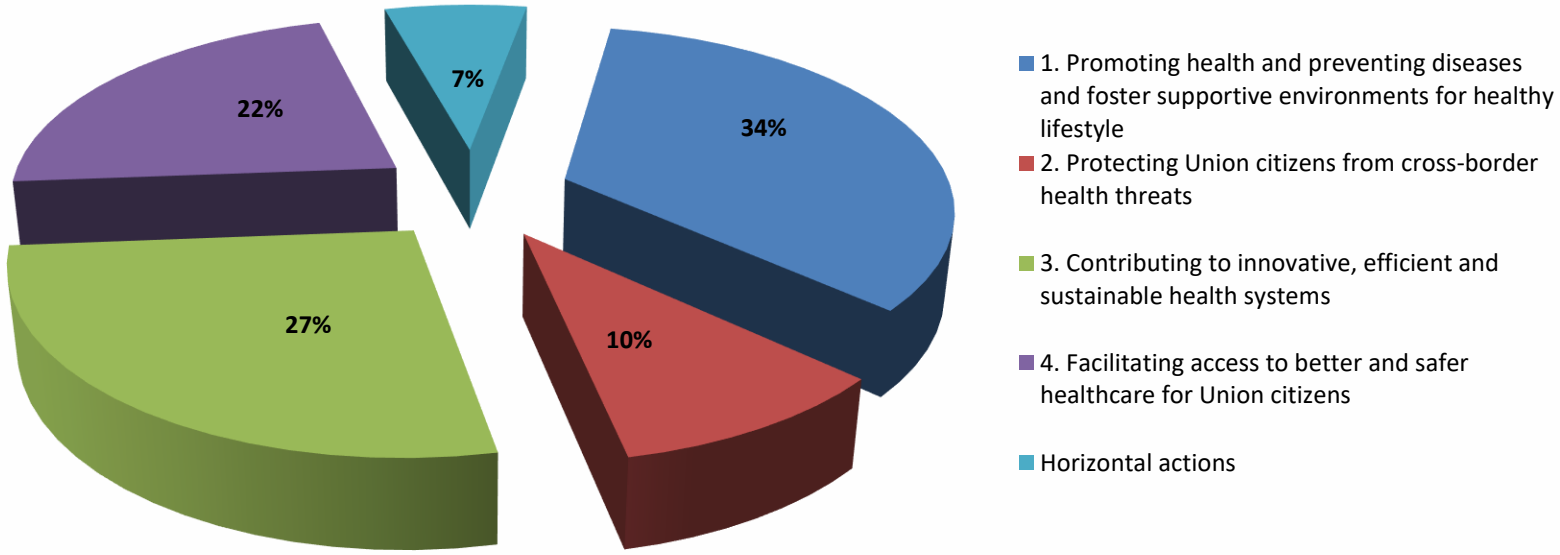
Objective 4: Facilitating access to better and safer healthcare for Union citizens

Budget allocation by objective 2014 - 2018



Main activities per thematic priorities

EU Funding: € 62 m

EURORDIS – European Organisation for Rare Diseases Association € 5 m

ERN- European Reference Networks (ERNs)
Coordination of ERNs € 26 m
Other activities € 3 m

Cooperation program with CoE/EDQM on specific matters related the improvement of safety and quality of blood components and tissues and cells for human application and dissemination of best practices
€ 0.5 m

Data 2014 - 2018

VISTART – Joint Action on vigilance and inspection for safety of transfusion assisted reproduction and transplantation
€ 2.3 m

GAPP – Joint Action on facilitatinG the Autorisation of Preparation Process for blood and tissues and cells € 1.2 m

JAMRAI – Joint Action on antimicrobial resistance and health care associated infections € 4 m

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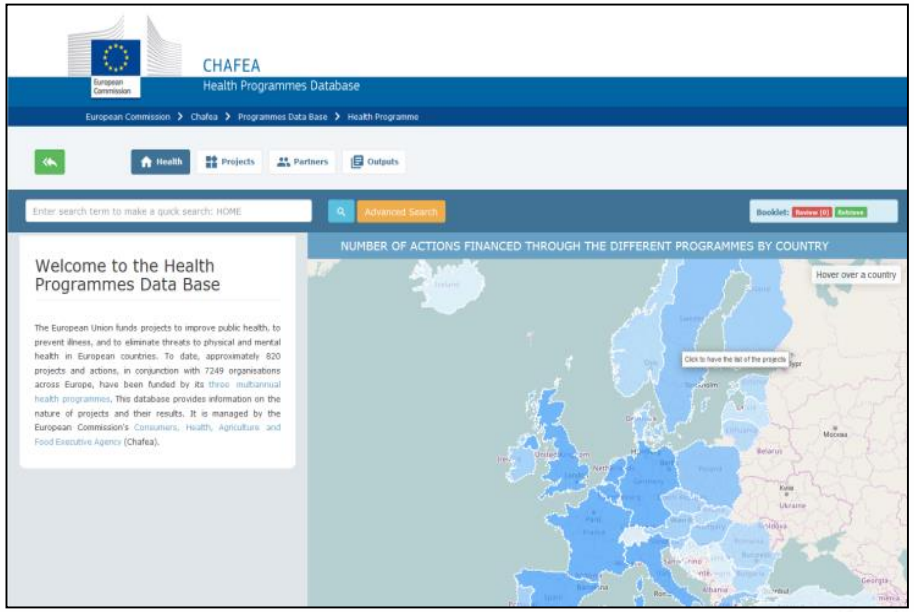
Useful links

European Commission Directorate-General for Health and Food Safety (SANTE) website
https://ec.europa.eu/health/home_en

Chafea Website
https://ec.europa.eu/chafea/health/index_en.htm

Chafea Project Database (2003-2019):
https://webgate.ec.europa.eu/chafea_pdb/health/



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European Reference Networks for Rare Diseases

European Commission

Irene Mathijssen

Erasmus University Medical Center, the Netherlands

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Facilitate access to better and safer healthcare for Union citizens'

Irene MATHIJSSEN
Coordinator ERN CRANIO, ERN Coordinators Chair
Erasmus MC Rotterdam, the Netherlands

The European Reference Networks on Rare Diseases

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ERN: content and objective

- Access for all European patients to expert centers
- Knowledge travels, not the patient
- Sharing knowledge between experts and patients

ERN: results

- Electronic platform for consultations with the experts
- European guidelines, developed with patients
- Sharing knowledge e.g. via webinars

ERN: uptake and follow-up

- At start, Western Europe and now inclusion of other MS
- In coming years, complete coverage of MS
- Upgrade of affiliated centers to full members

ERN: benefits for EU citizens

- EU citizen with rare disease can trace the expert centers and obtain information on diagnosis and treatment
- EU citizen can get a digital consultation via a local health care provider
- EU citizen can participate in ERN activities, including dissemination

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Rare diseases

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Yann Le Cam

European Organisation for Rare Diseases (EURORDIS)

European Commission

Session II_Objective 4: Facilitate access to better and safer healthcare for Union citizens

Yann LE CAM
Chief Executive Officer
EURORDIS – Rare Diseases Europe

Rare Diseases: Operating Grant, Joint Actions, ERNs

European Commission

Rare Diseases Community Actions: content and objective

Operating Grant:

- Consolidating the RD patient community
- Build capacity and empowering patient community
- Engaging patient community into implementation and monitoring of legislation and strategies

EURORDIS
RARE DISEASES EUROPE

Joint Action: priority health issues, deserving to be tackled at the EU level

- Improving health outcomes of people with rare diseases and rare cancers
- Enabling the implementation of recommendations of Expert Groups, harnessing the rare disease community

RD ACTION
DATA AND POLICIES FOR RARE DISEASES

European Reference Networks (ERNs):

- Contributing to build a EU wide infrastructure pooling knowledge and resources across Europe for highly specialised care
- Leading and supporting patient resources within and for ERNs

Rare Diseases Community Actions: results

Operating Grant:

- Strong and growing membership, outreach, dissemination in 7 languages
- Training activities consolidated in EURORDIS Open Academy
- Rare Disease Day, awareness raising campaigns
- Strong patient involvement in EMA activities & committees; ERNs and HTA activities

Joint Actions:

- Orphanet and OrphaCodes: standard common language and tools for health & research; go-to database
- Support to the establishment & development of ERNs (policy workshops, matchmaking tool)
- 15 EUROPLAN National Conferences & State of the Art
- Concrete recommendations prepared with and adopted by MSs for national uptake

European Reference Networks:

- EU flagship initiative with networks in 24 clinical areas
- Networks ERN Assessment Manual & Toolbox – EURORDIS led consortium
- Over 300 patients involved working together in 24 European Advocacy Patient Groups (ePAGs)

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Rare Diseases Community Actions : uptake and follow-up

Operating Grant:

- Membership reached 869 organisation, the community is increasingly complex, 40 national alliances, 68 federations
- Outreach and information dissemination targeted to membership sub-groups
- Scaling-up training for more patients and the depth of actions requires different areas and different levels of trainings

Joint Actions:

- 25 National Plans/Strategies for RDs are structured around same priorities in MSs, but require much more follow up & support
- Guidelines and technical recommendations, but still to be implemented in national policies based on best practices + new policy areas to be tackled
- OrphaCodes uptake has increased (over 11 MS), their value recognised – yet MS uptake has to increase, toolkits and guidelines are available

European Reference Networks:

- With 900 HCP Members in 300 hospitals, 26 EU MS, there is potential for uptake by more EU countries, full and affiliated members, sub-clinical groups so to increase disease and geographical coverage
- Scope for more extended patient involvement, with better anchorage in each ERNs and governing bodies (Boards, Steering Committees and TFs)

Rare Diseases Community Actions: benefits for EU citizens

Operating Grant:

- Structured, robust RD movement, with fair and objective representation of 25 million people in EU, and one of most vulnerable group in society
- Greater public awareness of RD and main promoter of RD policy
- Patient-centred RD policy and decision making in ERNs, HTA and EMA activities
- Competent, autonomous patient reps, for meaningful engagement in activities of common interest

Joint Actions:

- Reliable database, a common language for RDs for health information systems, with improved visibility of RDs
- Meaningful and effective policy making in the RD field, co-created by all stakeholders in the community
- Strong integration between EU policy and national policy, with EU cooperation fostering better national uptake and structural changes in national systems

European Reference Networks:

- Paradigm shift in healthcare delivery, planting the seeds of a EU-wide healthcare system in an area with high EU added value
- Cross-country sharing of knoweldge and resources with (potential) improved diagnosis and care RD patients
- Better patient involvement, overall ERN data strategy, integration in national HC systems

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Substances of Human Origin

European Commission

Paola Di Ciaccio

National Institute of Health, Italy

European Commission

Facilitate access to better and safer healthcare for Union citizens

Paola DI CIACCIO
Head of Foreign Affairs Division,
Italian National Transplant Centre, Italian National Institute of Health, Rome, Italy

VISTART and GAPP JAs: how the program has contributed to improve quality and safety of Substances of Human Origin

VISTART
VIGILANCE AND INSPECTION FOR THE SAFETY OF TRANSFUSION ASSISTED REPRODUCTION AND TRANSPLANTATION

GAPP

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VISTART and GAPP: content and objective

- ***Inspecting***: a common framework for harmonized targeted verifications of quality standards in Tissue Establishments for **blood, tissues and cells**

- ***Vigilating***: a shared approach to the notifying, assessing and investigating **serious adverse events and reactions**, disseminating the culture of safety and scanning horizon for new emerging risks

- ***Authorizing***: how to assess and authorize preparation processing in blood and tissue establishments, in order to ensure effectiveness and safety

VISTART and GAPP: results

- Inspection **guidelines** for EU Competent Authorities, common **training programme** for Inspectors, **joint inspection** programme

- Guidelines for **reporting SARE and identifying new risks** related to donation of SOHOs

- Approval of SOHO product processing:
  Seven regulatory principles

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Action: uptake and follow-up

- Ongoing discussions on a **common training program** for inspectors, as a tool of standardization of inspection methods. A proposal for common requirements **EU register** of international BTC inspectors has also been put forward: *under discussion in the Inspection Expert Subgroup chaired by EU Commission. All Member States.*

- VISTART supplied the basis for revision of official EU templates for **reporting of serious adverse events and reactions**, under Directive 2004/23/EU, as well as rapid alerts platforms for blood, tissues and cells: *under discussion in the Vigilance Expert Subgroup chaired by EU Commission. All Member States.*

- **Principles** for authorizing preparation processes: the basis for a devoted Joint Action (GAPP, 24 partners from 17 Member States) started in May 2018:
  - Good practice guidelines (plus 3 technical annexes) and platform for knowledge sharing

Action: benefits for EU citizens

- Inspections: Harmonization quality, training scheme, inter-MS scheme, support smaller countries
- Vigilance: A watchful awareness of what went wrong supplies precious information to avoid subsequent adverse events
- Authorization: proper pathway to tackle new products, gathering evidence at EU level, exchange of expertise, getting ready for the future

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Antimicrobial resistance

European Commission

Marie-Cécile Ploy

INSERM, France

European Commission

EU-JAMRAI

Joint Action on Antimicrobial Resistance and Healthcare-Associated Infections

Marie-Cécile PLOY
EU-JAMRAI Coordinator - INSERM

TOPIC: Antimicrobial resistance
ACTION: *Europe fostering synergies to keep antibiotics working*

European Commission

Action: content and objective

- **Tackling AMR:** 44 European partners and more than 40 international stakeholders working together to make EU a #BestPracticeRegion identifying and implementing evidence based measures to fight against AMR and HCAI.

- **Bridging the gap between declarations and actions:** We foster synergies to #KeepAntibioticsWorking producing concrete recommendations and promoting awareness and commitment by governments and stakeholders.

- **One health approach:** We work to ensure that all MS have a #OneHealth AMR strategy recognizing that human health, animal health and environment are interconnected.

Action: results

- Strengthening national and EU response against AMR
  - Mapping and assessing National Action Plans
  - Transforming EU through peer reviews: 9 country-to-country visits
  - Setting up a network of supervisory bodies
- Improving infection prevention and control
  - IDENTIFYING THE GAPS on implementation, research and communication
  - FILLING THE GAPS implementing pilot models (universal infection control framework) and ranking research gaps in infection control and prevention, in collaboration with the JPIAMR

Action: results

- Reducing antibiotic use: stewardship and surveillance
  - In human health (core components) and real-time surveillance system of antibiotic use and resistance
  - In animal health: European AMR surveillance network in veterinary medicine

- Raising awareness
  - #OneHealth butterfly effect: small changes can have large effects
  - Contest for an antibiotic resistance symbol

Action: uptake and follow-up

- Uptake:

- Follow-up:
  - **Integration**: adoption of the JA outputs at member states level (national, local, regional) into, for example, national policies (NAP), national actions or programs, etc.
  - **Sustainability**: strategy defining which elements/deliverables/results will be further developed, consolidated or run and by which entity/organisation this will/should be done
  - Involving Member States and Stakeholders

Action: benefit for EU citizens

- Increasing awareness on AMR
- Behavioural change
- Better implementation of infection control and prevention
- Sharing experience between all sectors (human-animal-environment)
- Providing evidence-based recommendations to policy makers and keeping AMR high on the political agenda for a better patient safety
- Reducing antibiotic use and antibiotic resistance burden

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European Pharmacopeia

European Commission

Michael Wierer

EDQM, Council of Europe

European Commission

Facilitate access to better and safer healthcare for Union citizens

Dr Michael WIERER
(Head of Medicines Division, DBO, EDQM/Council of Europe)

EDQM Grant:
OMCL Network and Biological Standardisation Programme

European Commission

EDQM grant: content and objective

- **Quality of Medicines**

Monitoring to ensure that EU citizens have access to medicines of appropriate quality

- **Manufacturer-independent Market Surveillance by testing**

Establishing and maintaining a network of Official Medicines Control Laboratories (OMCLs) that aims at work-sharing and optimised use of knowledge and resources

- **Standardisation of Biologicals**

Providing reliable tools for the testing of complex products

EDQM grant: results

Pillar 1: The OMCL Network provides

- efficient output during concerted routine surveillance test programmes
- ability to react rapidly and effectively in crisis situations (such as the Sartan case)
- mutual recognition of test results in Member States

Pillar 2: The Biological Standardisation Programme establishes

- methods and standards, embracing, where appropriate, the principles of Directive 2010/63/EU (Replace, Reduce, Refine the use of animals)
  - ✓ e.g. Acellular Pertussis vaccines safety test method:

  a variable animal test replaced by a more precise *in vitro* method

EDQM grant: uptake and follow-up

Pillar 1: OMCL Network

- Every year, about 30 OMCLs from EU/EEA MS participate in market surveillance testing activities and share about 1000 test reports for generic medicines
- Sartan case: Provision of scientific input, methods and analytical results for about 3800 samples
- Increasing recognition of the value of the Network among stakeholders

Pillar 2: Biological Standardisation Programme

- The new method for the Pertussis Vaccines included in the European Pharmacopoeia
- Legally binding as of 1/1/2020 in 38 Council of Europe member states
- To be applied by all manufacturers and OMCLs

EDQM grant: benefits for EU citizens

Pillar 1: OMCL Network

- The OMCL surveillance programme for generics is a reassurance for patients that generic medicines are of appropriate quality
- Sartan case: withdrawal of contaminated batches and support of the development of safe quality requirements

Pillar 2: Biological Standardisation Programme

- Common standards for quality control testing
- Biological medicines e.g. human plasma and vaccines can be independently controlled; appropriate quality is confirmed before they reach the patient

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