

# IMI1 Final Project Report Public Summary

**Project Acronym:** EUPATI

**Project Title:** European Patients'  
Academy on Therapeutic Innovation

**Grant Agreement:** 115334

**Project Duration:** 01/02/2012 - 31/01/2017



## 1. Executive summary

### 1.1. Project rationale and overall objectives of the project

Involving patients in research can hugely benefit the medicines development process: by bringing in their priorities and perspectives, patients can contribute to developing better treatments for them and others. Greater patient involvement in R&D will boost the efficacy and safety of new treatments and increase public support for medical research.

The European Patients' Academy on Therapeutic Innovation (EUPATI) was initiated to trigger a major rethink in the way patients and the public understand the medicines development process and their own involvement therein. Armed with a deeper understanding, patient experts and advocates will be empowered to work effectively with the relevant authorities, healthcare professionals and industry to influence the medicines development process for the benefit of patients.

The main objectives of the patient-led consortium, which includes patients' organisations, academic groups, NGOs and pharmaceutical companies were:

1. to develop and disseminate accessible, well-structured, comprehensive, scientifically reliable and user-friendly educational material for patients on the processes of medicines R&D, especially on end-to-end R&D processes, i.e. non-clinical R&D, clinical trials, personalised medicine, efficacy and safety assessment, risk benefit assessment, health economics, HTA and patient involvement in these processes.
2. to increase the capacity of "patient experts" and well-informed patients in patient organisations to be effective advocates and advisors in medicines research and development
3. to empower patients to provide appropriate patient-relevant advice and insight to industry, academia, authorities and ethics committees

EUPATI has generated educational resources in six key areas, namely

- Discovery of Medicines & Planning of Medicine Development
- Non-Clinical Testing and Pharmaceutical Development
- Exploratory and Confirmatory Clinical Development
- Clinical Trials
- Regulatory Affairs, Medicinal Product Safety, Pharmacovigilance and Pharmacoepidemiology - HTA principles and practices

Material has been developed in 7 languages (English, German, Spanish, Polish, French, Russian and Italian), serving 12 European countries with those native languages (UK, Ireland, Malta, France, Luxemburg, Belgium, Switzerland, Germany, Austria, Spain, Poland and Italy). With Russian, the

Patients' Academy reaches a large population within countries in central and Eastern Europe where a relatively large part of the population speaks or at least understands Russian.

The project addressed three audiences. The 'Expert Level' has delivered two English-language EUPATI Patient Expert Training Courses to patient experts, patient ambassadors and patient journalists. 96 advocacy leaders from patients' organisations have graduated from the course. More than 110.000 patient advocates have used the 'Education Level' material in the EUPATI Toolbox on Medicines R&D which is available in seven languages. The Toolbox includes resources in various formats such as texts, illustrations, slide decks, webinar recordings, glossary, and videos. In addition, video interviews and resources have been produced for patients and the wider public ("EUPATI Internet Library"), which guide patients, through the complexities of the pharmaceutical R&D process written in lay language and delivered in concise documents.

## 1.2. Summary of progress versus plan since last period

*[We have merged "Summary of progress versus plan since last period", and "Significant achievements since last report". "Major deviations from Description of Work" are covered in chapter 2.3]*

**Overall coordination** of the project (WP1, jointly led by the European Patients Forum and the private partners VFA and Bayer) has worked smoothly, with an Executive Committee ensuring close alignment between the work packages through monthly teleconferences and 3-monthly face-to-face meetings.

The **WP1 Communications Task Force**, led by EPF and GSK and comprising of representatives of all WPs, has focused on communications about EUPATI through newsletters and social media, such as Twitter, Facebook, LinkedIn and Google+. The EUPATI Network has more than 2.300 Facebook likes and about 4.110 Twitter followers, complemented by more than 2.000 followers of the Twitter channels of our National Platforms. The IMI project has posted more than 4.120 tweets.

The **three EUPATI Advisory Boards** (Project Advisory Board, Regulatory Advisory Panel and Ethics Panel) provided invaluable feedback and constructive criticism on project progress throughout the year and also gave expert input into content production processes, editorial review and public communication. The Ethics Panel has reviewed all submitted "Disclosure of Interests" forms and furnished crucial input on the recruitment procedures and eligibility criteria for the EUPATI Patient Expert Training Courses participants, provided input to Ethics-related topics of the course and to the EUPATI guidance on involvement of patients in ethical review.

WP2 conducted the **Final EUPATI Conference** on 14 December 2016 in Brussels with the opportunity to report on progress, but also to collect tangible feedback from all stakeholders about EUPATI's work. 214 participants attended, 120 of them being patient advocates, sharing experiences and developing new thinking about how to spur progress at the national level through EUPATI's National Platforms. In addition, WP2 organised the Graduation Ceremony for the Fellows of the EUPATI Course described below.

Great progress on **EUPATI National Platforms** has been achieved in 18 countries in 2016, more than the 12 countries that were originally planned in this project. National Platforms have been established in Austria, Belgium, Denmark, France, Germany, Greece, Ireland, Italy, Luxembourg, Malta, Norway, Poland, Portugal, Romania, Slovakia, Spain, Switzerland and UK. Additional National Platforms are currently in different stages of creation in Serbia and The Netherlands. The EUPATI National Platforms have organised and are preparing a variety of activities, including webinars, information days, mini trainings, and social media campaigns.

To increase the interaction between the members and gain more input from the EUPATI Network members, WP2 organized several **EUPATI Webinars** on the topics "Informed Consent for Vulnerable Populations", "Creating Trainings with the EUPATI Toolbox", "Revision of CIOMS Ethical Guidelines for Biomedical Research", and "Early collaboration - a recipe for solutions: drug development and treatment strategies may go hand in hand". Recordings are available online through the [eupati.eu](http://eupati.eu) website.

The second 14-month **EUPATI Patient Expert Training Course** was completed in December 2016. In addition to the e-learning part of the blended learning course, two face-to-face training events were held in Barcelona in March and September 2016. In an official ceremony on 13 December 2016, EUPATI graduated 96 Expert Patients from the first two EUPATI courses, coming from 31 different countries in 58 disease areas. The graduates have been awarded the title "Fellow of the European Patients' Academy" (in short: "EUPATI Fellow"). A survey among EUPATI Fellows showed that with their new expertise, their engagement as partners in all stages of medicines development has increased significantly - with the pharmaceutical industry, regulatory authorities (e.g., EMA committees), universities and HTA bodies. This collaboration ranges from advising researchers when starting new programmes in areas of unmet medical need, input on clinical trial design, and participation in the safety assessment of new medicines. Feedback of the first cohort of Fellows has been incorporated into the material and training programme for the second course. The results of a survey of Fellows are available, which outlines their learning and impact. The Moodle e-learning platform provided by WP5 has worked very effectively. The third Patient Expert Training course (post-IMI project) is currently recruiting. See <https://www.eupati.eu/eupati-training-course>.

EUPATI's second "core product", the **EUPATI Internet Toolbox on Medicines R&D, was launched in January 2016** ([www.eupati.eu](http://www.eupati.eu)). The EUPATI Toolbox covering almost all topics covered by the EUPATI course was provided in 7 languages after extensive reviews by the editorial board, supported by an external translation vendor. The comprehensive content of the EUPATI online resources are detailed in section 1.3 and are provided in at least seven languages, in line with the EUPATI mission. The educational material has been released under Creative Commons, meaning anyone can use, reuse and adapt the material for non-commercial purposes, just by crediting EUPATI and referring to the Creative Commons terms. More than 108.869 individual users have accessed the EUPATI Toolbox. Most users come from Europe (more than 76.000), but also from the Americas (18.000+). EUPATI users now extend to Asia with almost 10.000 visitors and to Africa with more than 2.700. 70% of our visitors to date are between the ages of 25 and 44. English content was most used (30.600 visitors), followed by Spanish (16.000), Italian (12.500), French (11.000), German (11.000), Russian (8.700) and Polish (7.200).

Following a 6-months public consultation process which gathered and incorporated hundreds of comments, in December 2016, WP7 published four **“Guidance Documents on patient involvement in R&D” covering ethics committees, health technology assessment bodies, regulatory processes and pharmaceutical industry-led medicines R&D**. Each guidance document recommends working methods and processes, and suggests specific activities and areas for patient involvement (<http://www.eupati.eu/guidances>).

**Sustainability** of EUPATI beyond the lifetime of this IMI funding period, despite many efforts in WP1 and WP7, was a key challenge. Since 2015, a well-considered sustainability plan was discussed, analysed and evaluated, investigating all different available options with and without IMI2 sustainability funding in intense discussions of the EUPATI Executive Committee Meetings and the EUPATI Advisory Board Meetings 2014-2016. Efforts to identify public funding sources from the EU or other public sources were undertaken but without tangible results. The launch of the IMI2 Sustainability / Exploitation call, which had been hoped to support the sustainability and further exploitation of EUPATI's project results, was postponed beyond the IMI funding period of the IMI EUPATI project. A "Bridging Model", called the "EUPATI Sustainability Programme 2017", based on approximately 25% of the annual budget of the IMI EUPATI project, combined with the full course content released under Creative Commons, was chosen as the sustainability model in 2016. The EUPATI 2017 Sustainability Programme is described as *a "Patient community-driven educational programme to maintain EUPATI throughout 2017-2019, focused on maximum exploitation of the EUPATI Patient Expert Training Course, on conduct of further EUPATI Patient Expert Training Courses, maintenance of the EUPATI Toolbox and the EUPATI brand, the EUPATI National Platforms, the EUPATI Fellow Alumni Network, the IT infrastructure, and on implementing updates of core EUPATI educational material"*. This approach ensures the continuity of EUPATI as a programme or even eventually an institution as well as a trusted brand for patient education in R&D while facing the realities of a postEU-funded project period and working towards longer term sustainability. EUPATI's primary focus in the 2017 Sustainability Programme will be the EUPATI Patient Expert Training Course and the EUPATI Toolbox on Medicines R&D as the core products of EUPATI in all available languages. Content will be updated to keep pace with external developments as necessary. The programme will further support the development of local trainings and "mini-courses" based on the EUPATI Toolbox by e.g. EUPATI National Platforms and pan-European capacity building programmes. The third EUPATI Patient Expert Training Course will begin in summer 2017, addressing the identified need to train more than the present number of patient experts/EUPATI Fellows. In a train-the-trainers programme supported by the preparation of "Toolbox Starter Kits" systematic training opportunities in various formats will be enabled in all EUPATI National Platforms to maximise the exploitation of the EUPATI Toolbox and to grow the knowledge of the health-interested public in the medicines R&D process. Efforts continue under the new Sustainability Programme to explore other means of sustaining EUPATI, through IMI2 and other pathways.

Overall, EUPATI has been very successful, has kept well on track and not only has delivered to plan and beyond objectives, but has substantially over-delivered.

### 1.3. Scientific and technical results/foregrounds of the project

EUPATI has developed various materials which are provided under the Creative Commons AttributionNonCommercial-ShareAlike 4.0 International (CC BY-NC-SA 4.0) license.

The English-language EUPATI Patient Expert Training Course has been released as an eLearning file set. The syllabus comprises of 103 topics in 6 modules and 81 online lessons, 178 optional further readings, 14 videos, 65 unrecorded quizzes and 6 assessments, complemented by the content of 2 x 4 days of face-to-face training material. Overall, it is structured in 6 modules:

- Module 1 – Discovery of Medicines & Planning of Medicines Development
- Module 2 – Non-Clinical Testing and Pharmaceutical Development
- Module 3 – Exploratory and Confirmatory Clinical Development
- Module 4 – Clinical Trials
- Module 5 – Regulatory Affairs, Medicinal Product Safety, Pharmacovigilance and Pharmacoepidemiology
- Module 6 – Health Technology Assessment (HTA)

The full course will be delivered again in the framework of the EUPATI Sustainability Programme in 2017, and is also available for download at <https://www.eupati.eu/download/>

The EUPATI Toolbox on Medicines R&D is available as open-access at [www.eupati.eu](http://www.eupati.eu) in seven languages. Across all languages the [www.eupati.eu](http://www.eupati.eu) platform contains 1463 articles, 254 presentations, 97 videos, 5 webinars, 246 images, 160 factsheets, 25 video interviews, 1 documentary, 148 infographics, 4708 glossary items and 2695 acronyms.

All additional foreground produced by the project is available as open-access on the [eupati.eu](http://eupati.eu) website in the “EUPATI Resources” section, e.g.

- Four EUPATI Guidance Documents on patient involvement in R&D
- Recordings of EUPATI webinars
- Presentations (PDF, video) of EUPATI workshops and conferences
- Publications (e.g., BMJ publications of WP3 data, and additional EUPATI publications in other journals)

### 1.4. Potential impact and main dissemination activities and exploitation of results

EUPATI has been a game changer for patient involvement in medicines R&D, and continues to be one of the **key drivers of the public debate on patient involvement in medicines R&D** on a pan-European level in all stakeholder groups including industry, regulators, academic researchers, ethics committees, HTA bodies and evidenced in the media and conference footprints that EUPATI had in 2016. (Main dissemination activities are described in chapter 3.2 “Key dissemination activities for the last reporting

period” and will not be repeated here). EUPATI has empowered European health-interested citizens and patient advocates, to become main actors in R&D. Academic and industry researchers have intensified discussions and implementation of patient involvement in their research activities (in addition through related activities like Patient-Focused Medicines Development, PFMD), and regulators have started to network on best practice in patient involvement in regulatory processes, following the EUPATI workshop held in Berlin in July 2016. Inter alia, EUPATI has increased the competitiveness of Europe and helped establish Europe as a preferred place for medicines research and development by developing into one of the most patient-centric R&D communities world-wide. With its educational resources on eupati.eu and its highly trained group of EUPATI Fellows, EUPATI has provided the self-growing, key resource for any kind of research that relies on knowledgeable patients, patient organisation representatives, patient advocates and patient experts.

The impact of the **EUPATI Patient Expert Training Course** has been huge, evidenced by the leading roles in the debate on patient involvement in R&D the 96 EUPATI Fellows have taken. A survey of experience and involvement of EUPATI Fellows 6-9 months after graduation, completed in December 2016, explored the longer-term impact of the course, documented the progress and impact of Fellows working as expert patients and gained insight on the value the course has added to the EUPATI Fellows’ work. Amongst other results, the survey, comparing activities before and after the course, revealed that the share of Fellows having a leadership role in patient organisations grew from 62% to 71%, those advising a pharmaceutical company grew from 13% to 44%, a regulatory agency from 21% to 42%, and advising a reimbursement / HTA body from 4% to 8%. For example, EUPATI Fellows engaged in advisory roles, acted as trainers, were involved in health policy advocacy, became speakers at conferences, started community advisory boards, assisted and advised other patient organisations, improved informed consent documents, reviewed trial protocols and contributed to trial designs.

When the IMI grant agreement was signed, the **EUPATI Toolbox on Medicines R&D** aimed to reach out to 12.000 patient advocates. By end of January 2017, the Toolbox had 108.869 unique users in 144.000 sessions; so therefore had a 10-fold higher outreach than initially planned. It has become a key knowledge resource and reference for any party interested in learning about medicines R&D. It is a testament to the impact of EUPATI and an indication of the value of the Toolbox that countries continue to request Toolbox translations (despite no EUPATI funding currently being available), with a number willing to fundraise themselves so these translations can be undertaken at the national level.

In addition, despite the initial lack of support from industry in a number of the 12 intended countries, and a relatively limited coordination budget, far more was achieved from the **establishment of the National Platforms** than was originally thought possible. Interest in developing platforms has also been received from outside the EU (e.g. Turkey, Brazil) which reflects the global impact of EUPATI.

With the EUPATI Sustainability Programme, this resource will gain further traction, as the number of EUPATI Fellows, users of the EUPATI Toolbox and training opportunities increases. EUPATI’s resources will spread more rapidly and broadly within the scientific community, the pharmaceutical industry, regulators as well as with healthcare professionals.

## 1.5. Lessons learned and further opportunities for research

One of the remits of the IMI Education & Training projects, like EUPATI, is to foster cross-stakeholder collaboration, not only to merge specific knowledge and expertise in the different areas and stakeholder groups involved in medicines R&D, but also to engage in and enhance collaboration between patient organisations, academia, industry, regulators and HTA bodies in the field of R&D, that can only radically change and improve by joining forces of everyone involved. From the latter it becomes clear that the public private partnership model is eminently well suited, if not the only viable approach, to achieve the objectives of such a project, and undoubtedly constitutes in itself an added value.

Given the diversity and different background, history and experiences of consortium members, it comes as no surprise that EUPATI got off to a fairly rough start in 2012, with all stakeholder groups having clear ideas about how “the industry”, “the patient organisations” or “the academics” operate and prioritize their activities, and what each parties’ interests are. However, it became apparent that many of these ideas were prevailing misconceptions and were not actually true in practice. Over time, based on very straight and clear ground rules for collaboration, on ethics, confidentiality and data privacy, laid in frameworks and terms of reference, and established in a multi-stakeholder governance structure, “camps” were replaced by task forces and teams that were focused on consultation, building consensus and jointly delivering work. Checks and balances through the Executive Committee, the Editorial Board, well-documented production and quality assurance processes, and the three Advisory Boards made sure rules and governance processes were being followed – and to flexibly deal with changing reality and adapt to needs that surfaced during the implementation of the project. It can be safely stated that a public private partnership (PPP), when executed as described, and even when populated by rather diverse stakeholder groups, provides the frame to develop a trustful, professional multi-stakeholder interaction, based on mutual respect and focused on the delivery of project results.

As a recommendation for future projects, based on research done in one of our deliverables (WP7 deliverable 7.1) and our experience in EUPATI, a few points may warrant consideration. In general, approaches to establish a PPP will have to be tailored to accommodate the specific needs, obligations, aims and/or formal restrictions of the involved stakeholders. In our opinion a number of principles for making a PPP work successfully are:

- Clearly specified, realistic and shared goals (reaching emergent milestones, achieving and measuring outcomes)
- Clearly delineated and agreed roles and responsibilities (getting the process to work in a clearly delineated framework that fosters collaboration)
- Distinct benefits for all parties
- Perception of transparency (within the PPP and within the public domain)
- Active maintenance of the partnership (build trust, mutual respect, openness)
- Equality of participation (commitment, ownership and responsibility of partners towards the partnership)



- Meeting agreed obligations (clarity of motivations, roles, capabilities and contributions, clear attribution of risks and blame)
- Gain recognition from others (communication within and to the public)
- Make best use of available and emerging technologies for communication, administration etc. to streamline processes and work most efficiently

In light of the experience gained with EUPATI, we recommend this model because most notably it is well established and operational since a number of years with formalised structures and can be easily adapted to a new PPP in the area of medicines development, public health or scientific research. It offers a clear, transparent and formalised governance model with defined responsibilities, interfaces and appropriate committees. EUPATI's structure of having 7 work packages aligned with the project goals, each with a public and industry co-lead, with patient organisations providing the project coordinator and the managing entity, has worked very well. Web-based collaboration tools have supported the process, even though in retrospect it would have been advisable to have an easier to use, central file storage space (cloud service) available from the start, which however, is thought difficult to introduce due to the variance of IT firewalls across pharmaceutical companies.

In terms of future research, Deliverable 7.7 describes a roadmap for the design and development of future topics inside and beyond the EUPATI Programme in more detail. In brief, this includes:

1. **Increase depth and breadth of EUPATI course content in identified topic areas** (e.g. National Competent Authority procedures, medical devices, personal and precision medicine, biosimilars and generics, real-world evidence, big data, registries, data ownership, vaccination, antimicrobial resistance, management of international trials, identification of comparators, primary prevention, etc.)
2. **Patient involvement and engagement in R&D and patient advocacy** (e.g. patient advocacy capacity building for patient engagement in R&D, support patient engagement in R&D – in all stakeholders, support development of specialist knowledge in specific vertical topic areas, development of a professional model of “Patient Advocacy Expert”, training on publications / article assessment / scientific publications / science articles in the media, training and support of patient advocates to lead/engage in other IMI2 and H2020 projects, matching advocates, opportunities and projects, etc.)
3. **EUPATI interaction and collaboration with other projects and initiatives** (e.g. partnerships, sharing knowledge and experience, advisory role and consultation on patient and public involvement)
4. **Increase innovation robustness** (e.g. assessment of the impact of innovation and new technologies on health systems, moving to an enabling HTA environment reflecting label changes more rapidly, better integration of technology and social systems innovation)
5. **Future ventures – e.g. in the form of Public–Private Partnerships** (financing models, reinvestment in research)
6. **Widening the EUPATI training audience to patients and carers at large** (addressing citizens, not only patients, addressing patients with low health literacy, addressing younger and older patients and citizens specifically).

7. **Widening the EUPATI training audience to other stakeholders** (e.g. pharmacists, nurses, physicians, psychologists, HCP students, industry staff in medical/ clinical/ legal/ compliance/ patient relations, policy makers, regulators/ assessors, and members of ethics committees)
8. **Geographical expansion** (e.g. USA / FDA sphere or other world regions; ICH, WHO and associated bodies)