

# policy impact

CSL Behring Public Policy Newsletter—Europe  
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## Healthcare Industry Issues Joint Paper on Industry Involvement in HTA

*Several healthcare trade associations including EuropaBio, the European association for bio-industries, have issued a paper on “The Value of Industry Involvement in Health Technology Assessment (HTA)”.*

In the paper, the healthcare industry calls for a robust and transparent framework in which HTA serves as a tool to encourage development of new and innovative technologies. These technologies will benefit patients and society by allowing healthcare planners to manage resources effectively and to appropriately fund healthcare. The paper also outlines the stages in the HTA process at which the industry can add value.

HTA is a multidisciplinary policy analysis that studies the medical, social, ethical, and economic implications of development, deployment and use of health technology. It is playing an increasingly important role in how national healthcare priorities are set and service provisions are delivered in most EU member states, influencing the level of market access.

In addition, the European Union is conducting a number of legislative and non-legislative initiatives, such as the development of an EU network of HTA agencies, the revision of the Transparency Directive and the Process on Corporate Responsibility in the field of Pharmaceuticals, which will impact the mechanisms of market access, including HTA methodologies and the procedural framework for pricing and reimbursement.

Because biotechnology medicines are complex and treat life-threatening and debilitating diseases, a holistic and transparent approach to HTA and market access is needed to ensure those products are appropriately valued at the national level. The broad objective is to ensure that all stakeholders, including the biotechnology industry, remain involved in the pan-European HTA and market access policy discussions. A platform is also

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### Patient Commitment

*CSL Behring is committed to saving lives and improving the quality of life for people with rare and serious diseases, worldwide. This commitment is reflected in the company's support of programs and activities for patients with rare diseases including bleeding disorders, primary immune deficiencies and Alpha<sub>1</sub>-proteinase inhibitor deficiencies.*



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## Expert Group Launches PID Recommendations in German Parliament

*The German Recommendations for Primary Immunodeficiency (PID) were developed by a panel of experts composed of patients, leading physicians and policy-makers led by two Members of Parliament, Mrs. Aschenberg-Dugnus, Member of the Health Committee and Mrs. Bracht-Bendt, Chair of the Children's Committee.*

The recommendations were officially launched in November. The document describes PID and the strategies that should be adopted to improve its diagnosis and treatment. Printed copies of the paper will soon be available from the German patient organization, Deutsche Selbsthilfe Angeborene Immundefekte e. V. (DSAI).

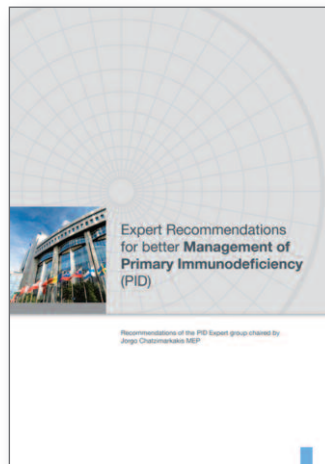
The German initiative builds on the work of a European panel of experts that prepared a set of high-level recommendations in 2010 for European

Member States on how to tackle PID. The Plasma Protein Therapeutics Association (PPTA) supports efforts to promote the recommendations across Europe. The European paper is available on the website of IPOPI, the International Patient Organisation for Primary Immunodeficiencies:

**[http://www.ipopi.org/uploads/media/news/Expert%20recommendations%20Mgt%20of%20PID\\_FINAL.pdf](http://www.ipopi.org/uploads/media/news/Expert%20recommendations%20Mgt%20of%20PID_FINAL.pdf)**

PID consists of a group of rare disorders, mostly genetic in nature, in which part of the immune system is missing or does not function correctly. Thus, people with PID are missing one or more of the vital weapons the body has against infection. PID is largely treated with therapies derived from human plasma, and in the most severe forms, with replacement of stem cells.

The rarity of these disorders often means that information on treatment options and access to care is not optimal. There have been previous initiatives at the EU level on the topic of PID that have raised awareness of the medical condition. The recommendations build on these to provide the appropriate basis and policy guidance for future decision-making in this complex disease state.



*Experts and elected officials gather for the German PID Recommendations launch meeting in Berlin. From left to right: J. Herborg, PPTA Germany; S. Ball, DSAI; N. Bracht-Bendt, MP; C. Aschenberg-Dugnus, MP; V. Wahn, Charité Berlin; R. Gatermann, PPTA Europe*

## EPPOSI Launches “Advanced Innovation Programs” for European Health Policy Development

*The European Platform for Patient Organizations, Science and Industry (EPPOSI) has launched four Advanced Innovation Programs on Chronic Conditions Management, Health Technology Assessment (HTA), Innovation in Healthcare and Rare Diseases.*

The HTA program will focus on the inclusion of societal benefits in the assessment/appraisal of new treatments while the program on Rare Diseases will be set up horizontally, i.e. as an integrated component in each of the other three programs.

CSL Behring is a member and partner of EPPOSI which is set up as an independent, not-for-profit, multi-stakeholder think tank based in Brussels. The Advanced Innovation Programs are designed to contribute to



*At the EPPOSI presentation of the Optimal Care Model*

European health policy-making by providing members and the wider public with high-quality independent research, capacity-building, knowledge exchange and dissemination with the aim of bridging the gap between innovation and improved public health outcomes.

Program content is agreed upon in accordance with EPPOSI's equally weighted governance structure among patient organizations, science and industry. Representatives from each stakeholder group work closely with the Secretariat to establish the strategic direction of the program and provide expertise to help shape research parameters and event content.

Further information on the scope and content of the programs as well as related activities and events are available at EPPOSI's website:

<http://www.epposi.org/index.php/programmes>



## EURORDIS Round Table of Companies (ERTC) analyzes “Compassionate Access to Rare Disease Therapies”

The European Rare Diseases Patient Organization (EURORDIS) held its 15th ERTC workshop in Paris in November on “Compassionate Access to Rare Disease Therapies.”



Compassionate access applies to situations where patients or their doctors know of a new medicine that might soon become available, but that has not yet completed the development or evaluation process. In these situations, the patient’s condition is likely to seriously deteriorate before the medicine becomes available.

Approximately 80 participants from patient organizations, industry and policy makers as well as representatives from regulatory authorities attended the workshop. They analyzed how compassionate use programs in Europe address the needs of rare disease patients. In particular, the participants discussed the effect of the present legislation on compassionate use in Europe, as well as actions to integrate compassionate use programs into an improved orphan drug development model. The model would be intended to better serve rare disease patients while taking into account the limited amount of data available on the medicinal product under development.

Several links to current EU dossiers and policy activities were addressed, e.g., whether the Health Technology Assessment of a product could be facilitated by the contribution of the data issued from a compassionate use program, or whether the Directive on “Patients Right to Access Cross Border Health Care” could provide guidance on access to compassionate use in a foreign country.

CSL Behring is an ongoing participant in the workshop series as part of our cooperative relationship with EURORDIS. The program and concept paper of the workshop are available at the EURORDIS website (as well as information on the previous ERTC events):

<http://www.eurordis.org/content/ertc-workshops>

### CSL Behring Public Affairs

CSL Behring Public Affairs improves patient access to care by providing decision makers with the information necessary to develop appropriate public policies. The public affairs department includes Dennis Jackman, Senior Vice President; Ruediger Gatermann, Director; Patrick Collins, Director; Ryan Faden, Manager, State Government Affairs; Mike Vogel, Manager, State Government Affairs; and Karla White, Manager, Public Affairs. Mr. Gatermann is responsible for European activities and has extensive public policy experience. He works closely with stakeholders and political thought leaders to affect change. Please contact him directly with questions or comments.

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being developed for EuropaBio members to exchange thoughts and interact with policy-makers, other trade associations, patient groups, payers and other relevant stakeholders, with the following key objectives:

- Focus on HTA for biotech healthcare
- Reward innovation in biotech healthcare
- Develop alternative and innovative access models.

The paper is available on the websites of the involved trade associations, e.g., via the following link to EuropaBio:

[http://www.europabio.org/sites/default/files/position/joint\\_healthcare\\_industry\\_paper-the\\_value\\_of\\_industry\\_involvement\\_in\\_hta.pdf](http://www.europabio.org/sites/default/files/position/joint_healthcare_industry_paper-the_value_of_industry_involvement_in_hta.pdf)