ecrd

26-28 May 2016 - Edinburgh

European Conference on Rare Diseases & Orphan Products

8th European Conference on Rare Diseases & Orphan Products

Game Changers in Rare Diseases

Delivering 21st century healthcare to rare disease patients: Together we can change the future!

EXECUTIVE SUMMARY

Organised by

EURORDIS
Rare Diseases Europe

Co-organised by

DIA DEVELOP INNOVATE ADVANCE

With the support of

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#ECRD2016

KEY FACTS AND FIGURES

MOTTO OF THE EUROPEAN CONFERENCE ON RARE DISEASES & ORPHAN PRODUCTS (ECRD)

- The European Conference on Rare Diseases & Orphan Products is the unique platform/forum across all rare diseases, across all European countries, bringing together all stakeholders - patients' representatives, academics, healthcare professionals, industry, payers, regulators and policy makers.
- It is a biennial event, providing the state-of-the-art of the rare disease environment, monitoring and benchmarking initiatives. It covers research, development of new treatments, health care, social care, information, public health and support at European, national and regional levels.
- It is synergistic with national and regional conferences, enhancing efforts of all stakeholders. There is no competition with them, but efforts are complementary, fully respecting initiatives of all.

PARTICIPANTS

- **800 attendees**
- **48 countries represented**
- **Over 120 session chairs, speakers and panellists**
- **28 sessions**
- **200 posters**

OBJECTIVES

- Disseminate the most up-to-date information related to the rare disease environment to all relevant stakeholders
- Demonstrate the importance of EU actions in the field of rare diseases and review progress made to date
- Elaborate strategies and mechanisms for developing further exchange of information between stakeholders
- Exchange knowledge and best practices on all relevant issues related to the rare disease environment

KEY POINTS OF SUCCESS

- The involvement of the **entire rare disease community**: patients' representatives, academics, healthcare professionals, industry, payers, regulators and policy makers
- The conference optimism and the opportunity for change by the identification of **game-changing policies and initiatives** in the areas of research, diagnosis, drug development, authorisation and access, care provision and social policy
- **Knowledge sharing of experience** from different countries including Australia, Austria, Belgium, Canada, Cyprus, Czech-Republic, Denmark, Finland, France, Germany, Ireland, Israel, Italy, Netherlands, Norway, Serbia, Slovenia, Spain, Sweden, UK and USA
- The transformational nature of the outcomes from ECRD 2016 will be to help **inform EU policies impacting on rare diseases** and to the Commission Expert Group on Rare Diseases; the EU Joint Action on Rare Diseases 'RD-ACTION'; the EU Health Programme; the EU Framework Programme for Research and Innovation 'Horizon 2020'

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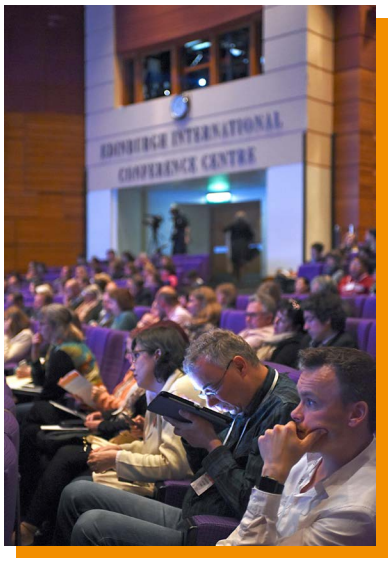
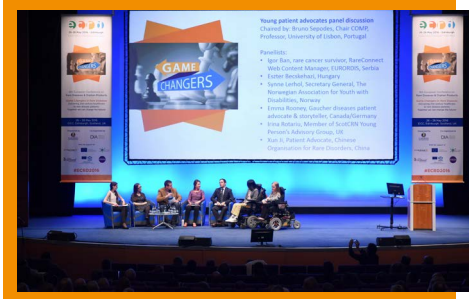
CONFERENCE OUTCOMES

ECRD 2016 was a pivotal conference for many reasons. Firstly, the presentations showed and celebrated how far the field of rare disease and orphan drugs has progressed since the first ECRD conference in 2001. Perhaps some of the strength of this work was the partnerships that sat behind the programme. These included but were not limited to partnerships with the European Medicines Agency (EMA) and in particular the COMP; The Food & Drug Administration USA (FDA) and in particular the OOPD; The Scottish Government; Orphanet; the European Society of Human Genetics (ESHG); Genetic Alliance UK; the European Hospital and Healthcare Federation (HOPE); the National Institute for Health Research (NIHR) RD Translational Research Consortium; EFPIA; EuropaBio and EUCOPE.



The conference optimism and the opportunity to implement even greater transformative translation for people living with a rare diseases came with the Opening Session, when three inspirational speeches set the scene (see below). The very exciting vision and obvious heartfelt personal commitment to the rare disease initiatives of Xavier Prats Monné, Director-General for Health & Food Safety at the European Commission, left a lasting impression on all delegates and proved to be a constant reference point in conversations across the entire meeting. His address provided a compass and navigational beacons in the field of rare diseases for those seeking to institute change and evoked the compassion and inspiration that comes from working with the families living with rare diseases.

From this perspective the transformational nature of the outcomes from ECRD 2016 will be to help inform EU policies impacting on rare diseases and to the Commission Expert Group on Rare Diseases; the EU Joint Action on Rare Diseases 'RD-ACTION'; the EU Health Programme; the EU Framework Programme for Research and Innovation 'Horizon 2020'



The European Conference on Rare Diseases & Orphan Products 2016 took place on 27 & 28 May in Edinburgh, Scotland, UK. The first day began with an opening and plenary session, followed in the afternoon by parallel sessions based on the 6 conference themes (as detailed in the following pages). On the second day, theme sessions continued before the conference concluded with a closing plenary in the afternoon.

OPENING SESSION

The opening session included a keynote address by Scottish Minister for Mental Health, Maureen Watts (whose much tweeted quote on rare disease patients set the tone of the conference - "It's not just What's the matter with you? but What matters to you?"), a speech from Alastair Kent, Director of the Genetic Alliance UK, and a speech from Xavier Prats Monné, Director General of the Directorate-General for Health & Food Safety, European Commission, said, "Our priority is and will continue to be supporting rare diseases and European Reference Networks".

PLENARY SESSION

The plenary session included a keynote from Tom Shakespeare from Norwich Medical School, UK and another from Igor Ban, rare cancer survivor and RareConnect Web Content Manager, Serbia. The plenary also included a forward-looking and inspiring young patient advocates panel discussion.

CLOSING PLENARY

In the closing plenary, theme leaders presented conclusions of the discussions during ECRD.

SPEAKER PRESENTATIONS

All speaker presentations have been added to the online agenda on the ECRD 2016 website (Friday and Saturday).

Videos of opening and plenary sessions, speaker presentations and posters available on the website: rare-diseases.eu

WHAT ATTENDEES SAID:

"There was a fantastic sense of community and I gained a lot from the experience of others"

"A wonderful, very informative meeting with great networking opportunities"

THEME SUMMARIES

THEME 1 | **GAME CHANGERS IN RESEARCH**

Developments in technologies for sequencing and bioinformatics continue to be the major game changer in research. The idea of moving from gene identification and diagnostics to therapy development is currently playing out in many rare diseases.

Within the sessions of the theme, participants addressed the move from research to diagnosis of these new technologies and considered the patient at the centre of new developments. There was also discussion around funding streams for rare diseases research. Participants also considered how the end game of rare diseases research (the delivery of new therapies to patients) can be enabled in a sustainable manner.

While speakers for theme 1 came from a range of clinical academic research bodies, patient organisations, clinical diagnostic service providers, health care professionals, industry, payers, regulators and policy makers, the key messages coming out of ECRD 2016 were resounding and exceptionally clear:

- Even when considering the great advances in clinical genetic diagnosis, the key issue still facing people living with a rare disease, and also health systems, is the **need for a diagnosis**, this being the portal to best care and treatment. Theme 1 had an obvious strong link to theme 2 on diagnosis.
- There is a **need for policy that protects and assists clinicians in sharing information to help patients achieve a diagnosis** (and health systems better manage rare disease) through cross-border and international efforts. Key pillars to enabling information sharing are ontologies such as Human Phenotype Ontology (HPO), including the Orphanet Rare Diseases Ontology (ORDO) and the Orpha disease classification codes.
- There is also a **need to develop precision public health policy frameworks**, within which the technological developments and information/ knowledge flowing from precision medicine initiatives can be supported and translated into public health systems - this is the transformative step to make these advances sustainable. This again highlights that it is not a question of diagnostics and technology, but of transformative integration of these diagnostic platforms into public policy.

In the area of rare diseases, clinical diagnostic services drive and open up the horizons for rare disease researchers, providing them with a rich field for discovery on disease pathways and novel targets for therapy. These insights feed back into the clinical setting.

THEME 2 | **GAME CHANGERS IN DIAGNOSIS**

Without a diagnosis families and the clinicians who support them are in the dark and possibly missing out on interventions that might improve their situation; the possibilities for research and development are limited; the development of therapies rarely gets off the ground; and the provision of truly integrated, multi-disciplinary and inter-institutional care seems virtually impossible to achieve.

A diagnosis allows a family to understand their situation, opens up access to interventions that may improve the prognosis for their child or affected family member, and allows them to build peer-to-peer support networks, rather than being pushed by external events over which they have no control.

This situation is changing rapidly thanks to progress in research. But much remains to be done before we can confidently expect every person living with a rare disease to speedily get a diagnosis.

The technological capabilities for diagnosis are developing at a rapid pace. However, experience of implementing these developments is still very low. Participants in theme 2 discussed the scientific, clinical, societal, ethical and practical questions that surround the issue of diagnosis, as well as to debate the best way of adopting these new technological developments.

- At ECRD 2016, participants who attended sessions of theme 2 explored the opportunities for delivering a diagnosis created by novel technologies such as whole genome sequencing and computer-driven phenotype recognition. Participants recognised the scientific advances made possible through sustained commitment to innovation and the delivery of the benefits of new knowledge to patient and family wellbeing.
- However, they also established that there are critical success factors that must be addressed if there are to be sustained health gains for patients and families affected by rare diseases - despite scientific and technological advances, there is still a need for investment in clinical service delivery. **Significant inequalities in access to diagnostic testing services still exist across Europe and the world**. Without investment in the development of quality assured genetic testing services, patients will struggle to obtain a diagnosis. There is an urgent need to support access to diagnostic services with integrated clinical care pathways and trustworthy information systems, which will reduce delays and help clinicians support families affected by a rare disease.
- Finally, participants in theme 2 concluded that we must accept that these are issues that can only be addressed through **collaboration across disciplinary, institutional and national boundaries**. No nation can go it alone in providing an across the board diagnostic service for rare diseases, much less deliver comprehensive and integrated care based on current scientific understanding and clinical best practice.

WHAT ATTENDEES SAID:

"I think the key success factors were the full cross section of the entire RD & Orphan drug sector but the second is the positive and enthusiastic attitude of all the participants"

"Important event to meet relevant stakeholders and develop new collaborations"

THEME SUMMARIES

THEME 3 | GAME CHANGERS IN DRUG DEVELOPMENT, AUTHORISATION AND ACCESS

Drug development, authorisation and access is no longer a linear path, but more of a cycle of evidence generation and review. Over the course of the past 15 years, since the adoption of the EU Regulation on Orphan Medicinal Products, opportunities for collaboration have been established at various different points on this cycle. Some of these are already working well, with a solid body of experience behind them. Others are in earlier phases of development.

Building on collaborative approaches that have delivered progress to date in the field of rare diseases, theme 3 participants considered which stages in the process need the most attention to ensure patients' access to the medicines they need.

Participants in theme 3 at ECRD 2016 came to the following conclusions:

- Regulatory systems are trying to keep up with the changes in drug development in terms of life-cycle approach and continuous data generation. In addition, flexibility, in particular for rare diseases, is constantly applied. The risks inherent to such a different approach are high as standards of regulatory decisions cannot be lowered, therefore **multi-stakeholder collaboration is key,** as well as high levels of engagement and responsibility. In order to ensure early patient access, innovative collaborative ways of developing and marketing medicines need to be developed keeping in mind patient benefit as the first objective. These need to allow for continuous data generation to inform decisions beyond market interests.
- There is an intensive way of approaching early access, with multiple initiatives by and around EMA and various HTA agencies, which have brought about pioneering collaborations with various forms of engagement. However, **this system is complex** and still incomplete because, although regulatory and HTA bodies get more and more involved, payers are not yet incorporated. There is also still a lot to be done on issues such as data gathering, so that the evidence addresses the needs of all stakeholders and decision makers in the process to access.
- While there is a set of potential 'game changers' coming into practice (e.g. the adaptive pathways), the field itself is rapidly moving **'ahead of the game'** with new challenges such as personalised medicine. Payers are also rapidly becoming more aware of challenges around access and sustainability, and are discovering the added value of collaboration in different fields such as horizon scanning, use of joint-HTA reports, information sharing and joint negotiations.
- So all in all, a significant level of interface clearly already exists across the 3 main stages of the pathway of medicines development (regulatory, HTA and payers). However, **further integration and collaboration is still required if we are to accelerate reimbursed access to rare disease treatments**.

Participants discussed how a new game is being played between companies, payers and patients who are burdened by mistrust, uncertainty and conflicting interests. In such a context, perhaps the biggest game changer lying ahead is for **payers across Europe to get better organised and present themselves as a more tangible and coherent partner**. They should weigh in on the debate on how to ensure sustainable access by making medicines effective and safe, but also valuable and affordable.

THEME 4 | GAME CHANGERS IN CARE PROVISION

Living in the 'connected generation', it is surprising to see that expertise in the rare disease field remains isolated. A wealth of knowledge and experience sit in pockets within Europe, locally accessible by only a scattering of people living with a rare disease.

Participants in theme 4 considered whether European Reference Networks (ERN), with the objective to connect the pockets of experts and highly specialised services across Europe so that the whole rare disease community can benefit, are the next big game changer in healthcare in Europe.

The rare disease community has been waiting ten years for the concept of an ERN (born in the 2011 directive on patients' rights in cross-border healthcare) to now be 'germinated' with the submission deadline for ERN applications in June 2016.

- Drawing on expertise from across Europe, participants shared case studies to show what the true potential will be in connecting isolated experts and sharing knowledge and information. To inform a view on **the true potential of ERNs**, they shared and discussed examples of how national and European networking has driven forward diagnosis, care, treatment and research. Creating **quality data** for natural history, epidemiology and outcomes of diagnosis, care and treatment was highlighted as a unique and critical feature of ERNs (where personal data is respected).
- Theme 4 participants understood the value of **clinical and patient outcomes** as a measure to demonstrate where one practice or treatment is superior to another. Clinical outcomes will be the common currency that enables experts to share knowledge and practices, driving improvements for all.
- ERNs will provide clinical services in a virtual environment through virtual European multi-disciplinary teams, which can enable the development of practices and treatments across Member States. Participants discussed concrete examples of how hospitals can connect up through a European IT Platform and examples of how clinicians run virtual European multi-disciplinary teams, their challenges and benefits. **Accessibility and interoperability is key to the success of these networks**. It is the responsibility of Member States to establish the links back to their respective healthcare systems, enabling each health system to be connected across Europe.
- Participants also discussed current barriers to finding the right expert, securing a timely and accurate diagnosis and their limits to accessing healthcare in their Member State or another. ERN network applicants were asked to **build their new networks addressing the practical issues that prevent patients accessing services**.
- Theme 4 participants already saw ERNs as the game changer in healthcare as ERNs have already changed the rules of the game. For example, Member State leads and clinicians are already collaborating instead of competing to share knowledge, experience and clinical practice. In addition, there is now more transparency on where the experts are and on the quality of care provided in hospitals. National clinical leads are breaking isolation of care by connecting experts, patients, clinicians and researchers, and anchoring these new networks into national health systems.
- Theme 4 participants concluded that there are high aspirations for ERNs, but we should not overload them with expectations. They must be given space to be effectively established in a simple way.

THEME SUMMARIES

THEME 5 | **GAME CHANGERS IN SOCIAL POLICY**

People living with a rare disease and their families face significant social and daily life challenges which affect their autonomy, their dignity and their fundamental human rights. Integrated care provision in coordination between health, social and local support services, via multidisciplinary care pathways and innovative care solutions, is a crucial game changer to tackle the unmet social needs of people living with rare diseases. Taking the new recommendations of the European Commission Expert Group on Rare Diseases to support the Integration of are diseases into social services and social policy, this theme looked both at the current policy scenario as well as at innovative care solutions from across Europe.

Theme 5 participants at ECRD 2016 concluded that:

- The perspective is changing - patients are "becoming" people with a rare disease, with complex needs, which require holistic care to support them in the full realisation of their fundamental human rights. As Synne Lerhol, Secretary General of the Norwegian Association for Youth with Disabilities, reminded us, **«People living with a rare disease don't necessarily define themselves by their disease»**.
- There is a need to **empower health services in their role to support the integration of rare diseases into social services and policies** by coordinating with social services, training non-healthcare professionals and developing information adapted to the needs of families and social professionals. The recently adopted Commission Expert Group Recommendations support health services to undertake this role and urge EU Member States to promote measures that facilitate multidisciplinary, holistic, continuous, person-centred and participative care to people living with a rare disease.
- With the ongoing development of European Reference Networks, there is the opportunity to revisit the criteria for Centres of Expertise. Some centres are already adopting a multidisciplinary approach, as is the case of Valle Hebron Hospital in Barcelona.
- Innovation is needed to improve care provision. As Anders Olauson, Founder and Director of Agrenska resource centre, commented «We often talk about bottom-up or top-down approaches, but **the solution for holistic care for people living with a rare disease is to work across»**, involving all stakeholders.
- Care needs to be **patient-centred, holistic and continuous.** It is essential to bridge the remaining gaps between health, social and local service providers. Coordinated and holistic care pathways, will make care more efficient, reduce the burden for people living with a rare disease and their families and bring about significant improvements to their autonomy and quality of life.
- Case management services have the potential support the development and implementation of holistic care pathways and make change happen.
- As Lene Jensen, Director Rare Diseases Denmark, concluded, «We need a revolution to move forward and meanwhile we also need to keep doing what we already do to support people living with a rare disease to deal with their current everyday challenges".

THEME 6 | **GAME CHANGERS IN GLOBAL SOCIETY**

Theme 6 drives home the message that rare diseases are truly global. This is the first time the ECRD devotes an entire theme to the global dimension of rare diseases. By connecting globally, we can accelerate advances in knowledge, public awareness and drug discovery and development, but most importantly we can connect patients to professionals, the public, and each other on an international level.

At ECRD 2016, theme 6 brought together key players forming the rare disease eco-system today. Panelists presented the patient, research and industry perspectives and explored the synergies between all stakeholders towards the common goal of more research, more treatments and better quality of life for people living with rare diseases. Theme 6 participants also debated how to make rare diseases as an international public health priority.

Theme 6 participants at ECRD 2016 debated discussed and concluded:

- Working together globally is essential to build a critical mass for holistic change in the field of rare diseases. Discussion revolved around the added value of greater synergy amongst the different stakeholders around the world to: accelerate research; give visibility to rare diseases in health systems; incentivise orphan drug development and approval; advocate for rare diseases in the UN system to provide an international framework to advance rare disease policies even in the poorest of settings.
- A lack of data in most parts of the world is one of the biggest hurdles. The Human Phenotype Ontology (HPO) is proof that international structures allow early dialogue and allows ideas to materialise. The challenge now is how to make it all meaningful in order to translate into diagnostic. **No country can do that alone.**
- **External pressure from the international community can be served to leverage patient interests at the national level**, especially in less developed countries. The claim of Universal Health Coverage is false if no strategy for rare disease is incorporated.
- The **global gap is growing between rare diseases in developed and developing countries** (high versus low/middle income). Technology, including biotechnology, genomics screening and testing, access to diagnosis, specialised care, and therapies (especially new drugs), and research investment are growing at uneven paces, while the disparity between the reality for rare disease patients in high-income countries and the real-life conditions for those in low and middle-income countries is increasing almost exponentially.
- There is a consensus that there should be a different trajectory for progress in **less developed countries**, in comparison to in developed countries. As we try and bring visibility to rare diseases in these countries we have to adapt and be ready for new challenges. The rare disease landscape is heterogeneous in all parts of the world. However, due to the Internet and social media, people are over connected and the expectations of patients are levelling out.
- International alliances are essential in every area but may be working more effectively at the research level (such as gene discovery and sequencing) and in terms of patient group collaboration (such as Rare Diseases International and facilitated through virtual communities such as RareConnect), than in other areas such as health policy, health services, and access.
- Global awareness - rare diseases have entered the global lexicon and discourse of ordinary people. Previously, the term was recognised in only a few environments. Now, even without a universal common definition, **there is shared understanding as to what 'rare disease' means**.
- **Theme 6 drove home the message that rare diseases are truly global – top down, bottom up and across all pillars, across the world.**

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- Ethical Medicines Industry Group (EMIG)
- European Federation of Internal Medicines (EFIM)
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- International Federation of Social Workers (IFSW Europe)
- International Pharmaceutical Federation (FIP)
- International Rare Diseases Research Consortium (IRDiRC)
- Istituto Superiore di Sanità (ISS)
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- Social Platform
- The Organization for Professionals in Regulatory Affairs (TOPRA)
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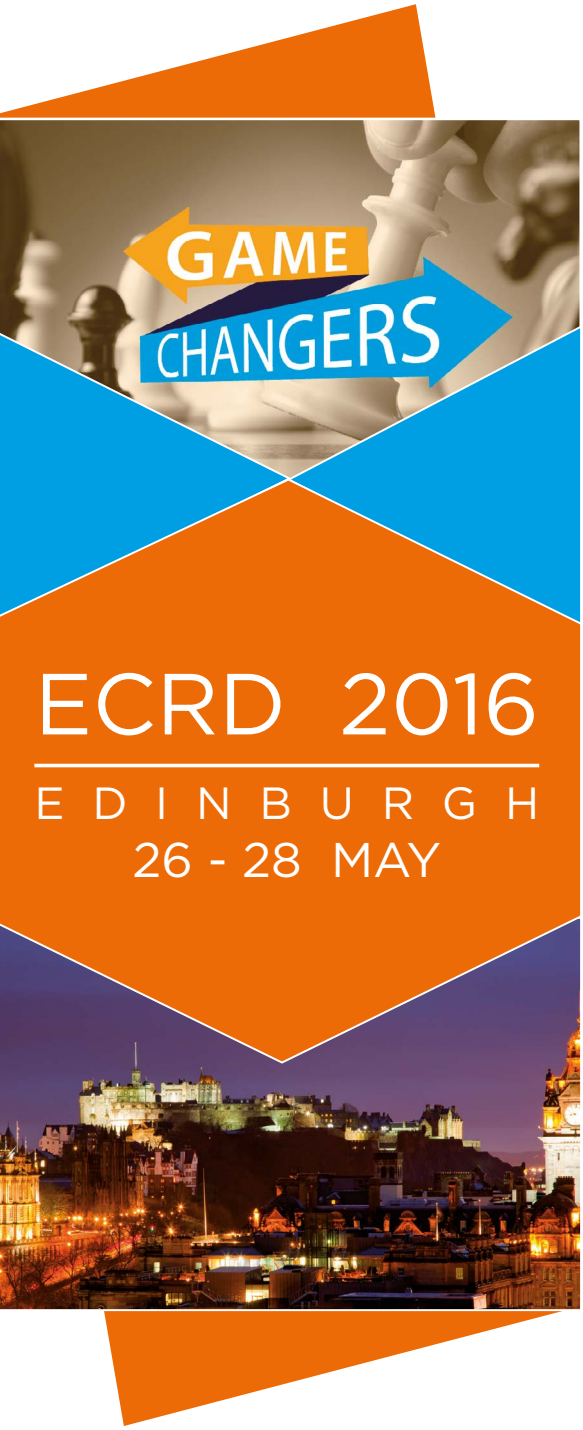

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ESHG European Society of Human Genetics

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Genetic Alliance UK Supporting. Campaigning. Uniting.

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Orphan Products Development

hope

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