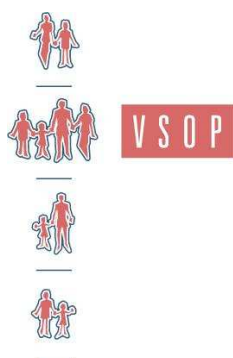


EUROPLAN National Conferences

CONFERENCE FINAL REPORT

THE NETHERLANDS



I. General information

Country	The Netherlands
Date & place	18-20 November 2010, The Hague, Hotel Eden Babylon
Website	www.vsop.nl
Organisers	VSOP (Dutch Genetic Alliance & Eurordis National Alliance): 18-19 November, Stuurgroep Weesgeneesmiddelen: 20 November
SC Members	<p>The already existing so-called 'Madurodam-group' functioned as the SC, consisting of:</p> <ol style="list-style-type: none"> 1. Mr. Dr. Cor Oosterwijk (Chair, Director VSOP) 2. Mr. Drs. Bert Boer (CVZ, Health insurance) 3. Ms. Dr. Carolien Bouma (NFU, Academic Medical Centres) 4. Mr. Prof. Dr. Martijn Breuning (LUMC, Geneticist) 5. Ms. Maud Creemers (Industry) 6. Dr. Gerard Engel (Forum Biotechnology and Genetics) 7. Ms. Drs. Marjolein De Booy (NPCF, Dutch Patient and Consumer Federation) 8. Ms. Prof. Dr. Martina Cornel (VUMC, Community Genetics) 9. Ms. Eugenie Dekkers (RIVM, National Institute for Health and Environment) 10. Ms. Drs. Jolanda Huizer (Steering Committee on Orphan Drugs) 11. Ms. Dr. Liesbeth Siderius (Patient representative and Paediatrician) 12. Mr. Dr. Cees Lips (Internist) 13. Mr. Herman ten Kroode (Medical Psychologist) 14. Mr. Drs. J. Crasborn (AGIS, Health insurance company) 15. Mr. Drs. J. Hofdijk (Advisor Dutch Ministry of Health) 16. Mr. Dr. John Roord (VUMC, Paediatrician) 17. Ms. Prof. Dr. Connie Schrandt – Stumpel (MUMC, Clinical Geneticist) 18. Mr. Jaap Schrandt (MUMC, Paediatrician) 19. Mr. Prof. Cees Smit (Patient representative) 20. Mr. Dr. Anton de Wijer (Board member VSOP) 21. Ms. Silvia van Breukelen (VSOP, Co-ordinator Rare Disorders) 22. Ms. Drs. José Willemse (VSOP, Policy Officer Rare Disorders) 23. Ms. M. Mann, advisor (Eurordis , Achse)

Workshops names, chairs, rapporteurs	<p>Early diagnosis (18 November):</p> <p>Chair: Dr. C. Oosterwijk (director VSOP)</p> <p>Speakers:</p> <p>Ms. Dr. G. Visser, paediatrician</p> <p>Ms. Dr. L. Siderius, paediatrician, patient representative</p> <p>Ms. Dr. I. van Langen, vice-chair Dutch Association for Clinical Genetics VKGN</p>
	<p>19 November:</p> <p>Conference chairs: Ms. M. J. Th. Martens, chair VSOP, former member Euro parliament and Dr. J.F.F. Lekkerkerker, chair Dutch steering Committee on Orphan Drugs</p> <p>Introduction: Ms. M. Mann, advisor (Eurordis , Achse, Ms. Drs. Jolanda Huizer (Steering Committee on Orphan Drugs) and dr. C. Oosterwijk, director VSOP</p> <p>Standards of care</p> <p>Chairs:</p> <p>Prof. Dr. C. Spreeuwenberg, former professor on integrated care (plenary introduction)</p> <p>dr. A. De Wijer, VSOP board member (rapporteur)</p> <p>Centres of expertise</p> <p>Chairs:</p> <p>Drs. W.J. Hofdijk advisor Ministry of Health (plenary introduction)</p> <p>Dr. C. Smit, expert patient, advisor Dutch Health Council (rapporteur)</p> <p>Financial aspects</p> <p>Chairs:</p> <p>Dr. A. Boer, board member CVZ (plenary introduction)</p> <p>Drs. J. Baars board member VSOP (rapporteur)</p> <p>Strategy national plan</p> <p>Chairs:</p> <p>Prof. Dr. P.W.J. Peters member Dutch senate, (plenary introduction)</p> <p>Dr. J.F.F. Lekkerkerker (rapporteur)</p>
	<p>Patient empowerment (20 November)</p> <p>Chair: Dr. J.F.F. Lekkerkerker</p>
Attachments	preconference paper, programmes, list of participants

Introduction

In the Netherlands, on the one hand the medical research and health care are at relatively high level compared to many other EU countries. For some rare disorders, research and care is a high level and most of the orphan drugs, (with a market authorisation and an approved reimbursement dossier), are fully reimbursed. Still, for many rare disorders, the situation urgently needs improvement. Patients experience a lack of coordinated care and integrated life-long care. Despite that, the government is not convincingly supporting the development of a National Plan, several initiatives in this area are not coordinated and the currently existing Dutch Steering Committee on Orphan Drugs ('Stuurgroep WGM) will no longer be financed by the Dutch government after 2011. In the light of this discouraging situation, VSOP had to combine and prioritize its role in both the national and European (Europlan) developments. Not all Europlan-themes could be dealt with *in extenso*. Priority was given to those themes that are urgent in the context of the national situation. Also was taken into account if a theme was, or had been, already under discussion elsewhere, since apart from VSOP¹, several other gremia are active on the national rare disease policy and education / information, like the Stuurgroep WGM, the Forum on Biotechnology and Genetics, the Dutch Orphanet, the Erfocentrum, etc.

The 18 November conference on early diagnosis was combined with national VSOP project on rare disorders, and at forehand, the number of participants was more limited and the 'on invitation only' policy was more strictly handled in comparison with the 19 November conference. On the evening of 18 November, a sponsoring dinner was organised for politicians and patient representatives in cooperation with BPRA, a public affairs consultancy, resulting in a donation for galactosemia research. The 19 November conference was as much as possible organised according to the Europlan recommendations.

¹ Within VSOP, more than 60 patients organisations are working together, supported by a 10-fte staffed office. Most of them are rare and genetic, or congenital. Many of them are working together within VSOP since issues related to the rarity of the disease is a priority of the organisation, others (also) recognize the emphasis on reproductive and ethical issues and the emphasis on medical research and the participatory role of patients in research. VSOP is well-recognized in medical, research and governmental policy bodies. Apart from VSOP, other more general Dutch patient umbrella organisations cover other aspects of health care and social aspects that may be of relevance for rare disease patients. Thus, although a lot is done, for historic reasons the rare disease community lacks a clear common face and a strong common voice, especially for the outside world, for the media and for 'new' rare disease patients. On the other hand, being less visible but profiting from working together on 'mainstream' developments in research and health care, including 'rare themes' may also be effective.

The 20-November meeting on patient empowerment was organised under the responsibility of the Dutch Steering Committee on Orphan Drugs ('Stuurgroep WGM). That day, a proposal developed in cooperation between VSOP and Stuurgroep WGM (who spoke upfront with several patient organisations about this proposal) was presented to strengthen and unify the voice of patients with rare disorders in the Netherlands. A patient working group was set up to access the support for the concrete elements in this proposal amongst patients and patient organisations for rare disorders. This investigation is still ongoing. Therefore, no recommendations from this morning session can be included in this report.

This report contains the recommendations that came out of the 18 and 19 November meetings. These were - as much as possible – formulated during the meetings itself. After the meetings, all participants had the opportunity to comment upon the draft recommendations. The – more descriptive – reports of the above mentioned meetings, including presentations and debates, are available in Dutch.

II. Main Report

Main Themes

Theme 1 - Methodology and Governance of a National Plan / Strategy (NP)

The following recommendations resulted from the 19 November workshop 'Strategy National Plan':

Aspects related to individual care:

- Pay attention to:
 - timely diagnosis;
 - improvement of timely reference to the right medical expert;
 - the (psycho-)social aspects of living with a rare disease;
 - transition of care during life-time, especially from paediatrician to adult medical care.

Policy aspects:

- The coordinating role of the Dutch Steering Committee on Orphan Drugs – ending itself as a governmental committee by the end of 2011 – must be continued to support the development of the National Plan and to quarantine the alignment of the actions of all national stakeholders. A separate commission, dedicated to speed up the development of the National Plan, could be an option.
- Stimulate a proper classification of rare diseases in the National Plan for Rare Diseases.
- Take care of the structured collection of data at both the national en international level.
- Formulate indicators to monitor the implementation of the National Plan.
- Guarantee the involvement of patient organizations in the development and implementation of the National Plan and support them in that role.
- The National Plan should be undersigned by all involved parties.
- The National Plan must connect to the national plans of the other EU member states.
- The National Plan must be future-proof. The government and policy-makers must pay more attention to the long-term, sustainability and should guarantee a coordination structure.

Financial aspects:

- The financing of the health care for rare diseases must be based upon standards of care, applied in centres of expertise.
- Health assurance companies should confirm their earlier expressed willingness to finance the health care for rare diseases based upon standards of care, applied in centres of expertise. This holds also for other stakeholders, like NFU (academic hospitals). VWS (Dutch Health Department), NZA (national health supervisory body) and CVZ (national body that determines what basic health care must be in the packets of the health insurance companies).

Finally, at the end of the 19 November meeting, it was concluded that anyhow, the development of the National Plan should just start, and should not be delayed any further by uncertainty about the position of the government and the mandate for whatever other stakeholder. Thus, in cooperation with other stakeholders, like patients, the Stuurgroep Weesgeneesmiddelen will start to prepare the National Plan. Several national meetings will follow to contribute to its development.

Theme 2 - Definition, codification and inventorying of RD

This theme was not addressed separately.

Prof. Paul Peters – member of the Dutch senate, appointed on an academic endowed chair by VSOP and working for the European Commission in Luxembourg as an expert on rare disease from 1995 to 1998 - was involved in the first phase of European policy on rare diseases. Since for most rare disorders, the real prevalence is unknown, he advised to use a more functional definition, taking into account the actual situation of the disorder, weighing unmet medical needs and the burden of the disease.

In the early diagnosis meeting it was stated that there are inadequate data on rare diseases, insufficient the access to the existing data, problems with validity and codification.

In the recommendations on the other themes, remarks related to an 'inventorying of RD' mainly concern the plea for a national database on rare disorders.

Theme 3 - Research on RD

This theme was not addressed separately.

In the recommendations on the other themes, the importance of European and international cooperation was stressed, as well as the necessary link between research and care.

Theme 4 - Standards of care for RDs - Centres of Expertise (CoE)/ European Reference Networks (ERN)

This report on theme 4 contains the recommendations that came out of the two 19 November workshops 'Standards of care' and 'Centres of expertise', as well as some recommendations that came out of the 18 November meeting on early diagnosis.

It is important to notice that Standards of Care (Dutch: 'zorgstandaarden') have a special place in the Dutch health care system. The patient-experience based opinion on good care for the disorders is an important basis for the development of the standard. Subsequently, a Standard of Care may develop into a prerequisite for health insurance companies to pay medical professionals for their services to patients. At this moment, only a few of such standards of care exist, and only for common disorders. Currently, VSOP is developing Standards of Care for, and with, 15 (patient organisations for) rare disorders. Were possible they are connected to 33 standards of treatment for rare disorders that are developed by VSOP for general practitioners.

Standards of care

Aspects related to individual care:

- Standards of care must stimulate the personal involvement of the patient with his/her treatment as well as his/her ownership of medical data in a personal health record.
- By definition, standards of care must be holistic and be multidisciplinary and integrated of character, including family elements, transition, self-management, revalidation, psychological care, etc. Also other policy instruments should stimulate this holistic approach.
- Standards of care (and financing elements, like DBCs – Diagnose-Treatment Combinations) must include care from cradle to grave.
- Health professionals must be stimulated to take up a more responsible role in the organisation of care for the individual patient.

Policy aspects

- Apart from aspects related to individual care that are the main part of the standards of care, standards related to the national coordination and organisation of the health care related to a specific rare disorder, are as important. These elements should therefore be worked out in relation to each other.
- The current Dutch meta-standard of care should be worked out, with flexibility, in general and disease-specific modules.
- Best practices must be exchanged in a structural way.
- In the process of developing consensus-based standards of care, the interaction between patients (organisations) and medical professionals must be facilitated, if needed with the help of an independent person or organisation.
- Pay attention to, and leave room for, both diversity and minorities.
- Create a coordination point or platform for the realisation, authorisation, implementation and evaluation.

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- Standards of care should be evaluated and updated by both patients (organisations) and medical professionals.
 - When developing standard of care, don't forget the international context and pay attention to foreign standards of care and foreign disease experts.

Financial aspects

- A national framework for the financing of standards of care must be developed
- Integral care must also contain elements of knowledge-management.
- Make (the financing of) medical (orphan) devices and (orphan) drugs part of the standards of care to guarantee both continuity and the right context of use.

Early diagnosis

Early detection of rare disorders in children:

- Investigate the introduction of a personal health record, starting at birth or pregnancy, to be used for timely recognition of a rare disorder at early age. Both medical and data based on personal experiences may be included.
- Diagnostic tools must be developed that help medical professionals in primary health care to recognize a (clusters of, common symptoms of) rare disorder(s) in children
- Parents should have the opportunity to have their 6-12 months old child seen for rare disorders. This may include invasive screening for some severe disorders of childhood that are currently not (yet) part of the Dutch neonatal screening (for example, the absence of a proper therapy is a current exclusion criteria, as defined by the Dutch Health Council).
- If one suspects a child to have a rare disorder, regional multidisciplinary services must be available for diagnosis and subsequent referral to a centre of expertise (similar to the formula of the Dutch Sylvia Tóth Centrum, located in the Academic Hospital Utrecht). Travelling multidisciplinary teams and/or video consulting via the internet could also be considered.
- A cost-effectiveness study, or pilot, investigating the above mentioned options with regard to early diagnosis in children should be performed.

Early detection of rare disorders in adults:

- Rare disorders must be registered in one national database, linked to more detailed medical database at a national level, and also linked to (less detailed) international databases. Data-sharing options must be included.
- The infrastructure needed for cascade-screening of (rare) genetic disorders that can be prevented or treated, must be financed at a national level. Currently, only the cost for the individual genetic testing are reimbursed.
- Medical professional in first and second-line health care must be educated regarding referral to genetic services and participation in family and cascade-screening of (rare) genetic disorders.
- One should anticipate on the rapid developments in the field of whole-genome analysis, amongst others by the timely development of guidelines.
- Multidisciplinary cooperation and integral care must be stimulated.

Centres of expertise

Aspect of individual care

- Centres of expertise should deal with integral care, including social.
- In an expertise network, diagnosis should be centralised, treatment may be organized otherwise.

Policy aspects

- Realise an infrastructure for the centres of expertise that guarantees financing, cooperation, knowledge-management and continuity.
- The development of centres requires national coordination. This should not be in the hands of the academic hospitals or their umbrella organisation (NFU) themselves.
- Medical professions within centres of expertise should in the first place feel responsible for quality monitoring, also towards each other. In addition, quality control of the centres must involve the patient, the government, the hospital and the health assurance companies.
- A central certification of the central must be set up.
- Each centre must:
 - Work multidisciplinary;
 - Combine care with research;
 - Be a contact point for patients, parents, families;
 - Cooperate with patient organisations concerning governance and treatment policy.
- All centres must be connected to a national database for registration of, amongst others, symptoms, diagnostic and treatment parameters.
- Medical investigators and doctors must be stimulated to bundle their data in this national database and couple these data to both individual treatment plans and international data.
- Realise a centre or service for the ultra-rare disorders that are too rare for their own, independent centre of expertise.
- The centres should also deal with the national/European standardization of laboratory diagnostics.
- Stimulate centres dealing with different disorders to exchange on symptoms, complaints, etc.
- To stimulate the R&D of orphan drugs, one should seek for ways to give biopharmaceutical companies controlled access to patient data in expert centres and the centres to industry data.
- To exchange expertise, national centres of expertise must always be connected to European reference networks.
- Make better use of the internet to exchange data and for diagnosis by other (foreign) experts.

Financial aspects

- Make use of the Dutch law concerning special medical treatments (WBMV, Wet Bijzondere Medische Verrichtingen) to finance the centres.
- The infrastructure that is needed for structural cascade screening of several (rare) genetic disorders must be financed at a national level.
- In the (near) future, health assurance companies must only finance (treatment in) centres that work according to the quality standards that are currently developed (amongst others by the Stuurgroep Weesgeneesmiddelen)

Theme 4.1. Orphan Drugs (OD)

This theme was not addressed separately. An extensive description of the situation regarding OD in the Netherlands was recently submitted to Eurordis in the context of a Joint Survey on Patients' Access to Orphan Medicinal Products in Europe.

Theme 5 - Patient Empowerment and Specialised Services

The 20-november meeting on patient empowerment was organised under the responsibility of the Dutch Steering Committee on Orphan Drugs ('Stuurgroep WGM). That day, a proposal developed in cooperation between Stuurgroep WGM and VSOP was presented to strengthen and unify the voice of patients with rare disorders in the Netherlands. A patient working group was set up to access the support for the concrete elements in this proposal amongst patients and patient organisations for rare disorders. This investigation is still ongoing. Therefore, no recommendations from this morning session can be included in this report.

Theme 6 – Sustainability

This theme was not addressed separately.

Theme 7 - Gathering expertise at the EU level

This theme was not addressed separately.

Conclusion of the Final Report

EUROPLAN Recommendations and EUROPLAN Indicators for the advancement of a national strategy in the country are very useful. However, there should be sufficient freedom to make the recommendations and indicators work within the national context.