

STRATEGY OF THE NETHERLANDS IN THE FIELD OF RARE DISEASES

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Introduction

This document presents an overview of the government's strategy in the field of rare diseases and orphan drugs.¹ The government, and in particular the Ministry of Health, Welfare and Sports (VWS), has been involved in this field nationally and internationally since 1995. Initially, participation was limited to the Council Working Party of the European Council of Ministers regarding the proposal for a Regulation for orphan drugs and to discussing, with all relevant stake holders in The Netherlands, how to develop and implement policies in the field of rare diseases. Following an advice of the Council for Health Research, the Minister of Health, Welfare and Sport, installed in April 2001 the Steering Committee on Orphan Drugs as part of the national strategy. The year 2001 can, therefore, be considered as the formal start of the implementation of the government's policies in this area.

Several years have passed and although improvement for some patients with a rare disease has been attained, the situation for many others is still far from optimal. There are several reasons for this, some of which are the following. Although since the year 2000 nearly seventy orphan medicinal products were authorized in the European Community and although ten times as many products were granted an Orphan Designation, the number of rare diseases for which no drug will be available in the near future is several times larger.² Knowledge about disease mechanisms, crucial for developing medicines, is still insufficient in many rare diseases. Knowledge about the natural history of many rare diseases, essential for the optimal treatment of patients, is also often inadequate and this is as true for rare diseases that can be treated with orphan medicinal products as for diseases for which no orphan drugs are available. Moreover, the available, and possibly useful, information for patients with a rare disease is often not easily accessible. Nevertheless, in comparison with some years ago, there is greater awareness and understanding among physicians and other care providers and among relevant social, political and scientific communities for the problems that patients with a rare disease encounter. Greater awareness and understanding can also be observed among representatives from pharmaceutical industry, health insurance companies and the media. In spite of these improvements, the situation remains far from optimal for many patients. The European Commission, aware of the differences between member states, consulted the member states and many other stake holders, via an open consultation process, on how to improve the situation of patients with a rare disease. The Ministry of Health, Welfare and Sport endorsed this initiative and responded to it.³ The outcome of the consultations led to the Council Conclusions of June 2009 with respect to rare diseases and a Recommendation to Member States.⁴ The Netherlands accepted the Conclusions and Recommendation. An important issue in the Recommendation is that Member States should have developed by the end of 2013 a national strategy (or a national plan) with respect to rare diseases. Each Member State can choose how it will devise and implement its strategy, taking into account its actual situation with regard to rare diseases. It is clear that, in spite of many similarities between Member States, for example a broadly accessible social healthcare system and the availability of authorised orphan drugs, there still exist significant differences between Member States. These differences may lead to different strategies in the Member States.

¹ The minister of Health, Welfare and Sports, Mrs. E. I. Schippers, sent a letter including annexes to Parliament on 29 February 2012 (Parliamentary papers II, 2011-2012, 29477-184) regarding the Dutch national strategy with respect to rare diseases. This letter to Parliament is at the basis of the future strategy of the Netherlands.

² The number of rare diseases worldwide is not known, estimates vary from 5000 to 8000. A sponsor can submit to the European Medicines Agency an application for Orphan Designation for its medicinal product before the product is market authorized. The European Commission will grant the Orphan Designation under the condition that the criteria, as laid down in Regulation (EC) 141/2000, are fulfilled.

³ Letter to DG SANCO, 14 February 2008.

⁴ Council Recommendation of 8 June 2008 on an action in the field of rare disease (2009/C 151/02).

General

The main objective of the strategy is to improve the health of patients suffering from a rare disease. Patients with a rare disease are entitled to the same quality of care as patients with a more prevalent disease. Experiences of the past years have shown that improvement is attainable. Governments are in a position to make improvements in general areas such as quality and accessibility of healthcare, to promote research, and to screen for - and in certain cases to prevent - rare diseases. A government can, via targeted subsidies to relevant parties, help resolve certain specific problems. Governments have generally, however, no direct influence on the individual relationship between a patient and his doctor (or another healthcare provider) or on the interaction between the patient and the healthcare institutions. It has a limited influence on the price of orphan drugs.

Since 1995 the Ministry of Health, Welfare and Sport (hereafter: the Ministry) has via the Council for Health Research (*RGO*) consulted with relevant parties (or stake holders) in the field of rare diseases. These parties included patients, healthcare providers, researchers, health care insurers, pharmaceutical companies, and independent authorities such as the Health Care Insurance Board (*CVZ*) and the Medicines Evaluation Board (*CBG-MEB*). In 1998 the Council for Health Research presented a recommendation regarding rare diseases and orphan drugs.⁵ This recommendation provided an important contribution to the development of the Ministry's policies. Following this report, the Ministry initiated a national strategy with regard to rare diseases and orphan drugs and provided funding to implement this strategy. One very important aspect in the strategy of the last decade was the Steering Committee on Orphan Drugs (hereafter: the steering group). This steering group, installed in April 2001, functioned very well, but was nevertheless disbanded on 31 December 2011 for reasons that will be explained below. Most tasks of the steering group were devolved to the various stake holders in this group. Some remaining tasks were devolved to The Netherlands Organisation for Health Research and Development (*ZonMw*). Notwithstanding these changes, the Ministry will remain committed to rare diseases and orphan drugs both at the national level and the international (mainly European) level.

Before explaining in more detail the future strategy of The Netherlands, it is helpful to have a look at the strategy of the past years and in particular at the Steering Committee on Orphan Drugs. The future strategy will be built on past experiences.

⁵ *RGO* (Council for Health Research) recommendation no. 16. Recommendation on Orphan Drugs, 1998. *RGO* is an integral part of The Health Council of The Netherlands since 2008.

The Steering Committee on Orphan Drugs

The government, following preliminary discussions with relevant parties (see Introduction), made an important stride in 2001 in the area of rare diseases and orphan drugs by creating the Steering Committee on Orphan Drugs. All relevant parties were invited to participate: the Dutch Federation of Patients and Consumer Organisations (*NPCF*) in co-operation with the Dutch Council of the Chronically Ill and the Disabled (*CG Council*), the Association of Collaborating Parent and Patient Organisations (*VSOP*)⁶, physicians and caregivers, researchers, two umbrella organisations of pharmaceutical industry (*Nefarma* and *Biofarmind*), the umbrella organisation of the health insurance companies (*Zorgverzekeraars Nederland*), the Medicines Evaluation Board (*CBG-MEB*) and the Health Care Insurance Board (*CVZ*). The remit of this steering group included: making an inventory of on-going activities in the field of rare diseases, coordination and stimulation in a variety of areas (healthcare, research, information to patients and other relevant stakeholders, and international coordination with other stake holders). The steering group operated independently and was supported by a secretariat within The Netherlands Organisation for Health Research and Development (*ZonMw*). The steering group played an important role in providing information to the relevant parties and individuals with regard to reimbursement of orphan drugs (helping to develop the policy rule on reimbursement of orphan drugs) and in developing a proposal for a research program at ZonMw. The steering group was a partner in international discussions with similar groups in other states in Europe and elsewhere.

Following the creation of the steering group in 2001, circumstances in the field of rare diseases and orphan drugs however, gradually changed. Stake holders in the steering group acquired more expertise in their own particular field. This gain in expertise is evident among all parties that held a seat in the steering group. Another important development was the gradual change in focus from the scarcity of orphan medicinal products to problems related to the care of patients with a rare disease. These developments initiated discussions between the stake holders in the steering group and the Ministry on how to proceed after the year 2011. The outcome of these discussions was that the Ministry and the steering group agreed to disband the steering group and to continue activities in a different manner. The stake holders would continue activities in their own specific field, while at the same time liaising and cooperating with other stake holders if necessary. Activities outside the expertise of the stake holders, but nevertheless important, would be devolved to ZonMw in this new situation (see next section).

⁶ The Association of Collaborating Parent and Patient Organisations (*VSOP*) is a collaborative [effort] of currently about 65 patient support organisations, most of these for conditions of a rare, genetic and/or congenital nature. "The Association of Collaborating Parent and Patient Organisations" is an unofficial translation of the association's Dutch name.

A national strategy for the next years

The current strategy will be partly continued and partly modified in the next few years. Even though the past strategy certainly produced a number of important results, it is evident that some changes in strategy have to be made nonetheless because of changing circumstances occurring the past ten years.

The policy regarding the steering group was altered in January 2012. Most tasks were devolved to stake holders in the former steering group and some remaining tasks were devolved to The Netherlands Organisation for Health Research and Development (*ZonMw*). These tasks are within the remit and expertise of *ZonMw*. Extra funding for the stake holders is not foreseen in the new strategy, with the exception of the funding of some projects by patient organisations. The Ministry has however, with the remaining tasks in mind, allocated specific funding to *ZonMw*, (€ 4 x € 100,000 supplemented with € 480,000 for the years 2012-2015).⁷ *ZonMw* will have set up a specific secretariat supported by a feedback group consisting of representatives from the stake holders. Among the tasks of the secretariat are collecting and streamlining information from relevant parties or individuals with the aim to help implement the Ministry's strategies and policies or the policies proposed by the various stake holders, either directly or via the website (www.nationaalplan.nl). *ZonMw* will also facilitate a number of projects such as strengthening the patient's voice, establishing an information desk for patients and improving medical and social care of patients with a rare disease. These projects will be in consultation and cooperation with health care providers and patients.⁸ The secretariat will help to formulate a strategy for the years after 2015. Last but not least, the secretariat will be part of the international network of similar organisations and participate in additional relevant international activities.

For a number of years there existed already some collaboration between the steering group and the Biotechnology and Genetics Forum (*FBG*), a forum also subsidised by the Ministry (currently €125,000 annually from 2012 up to 2015). The Ministry delegated the former steering group's 'forum function' (i.e. the plenary sessions between the relevant parties) to the *FBG*. General or specific issues in the field of rare diseases and orphan drugs will be on the agenda and if necessary *FBG* will advise the Ministry.

In summary, the steering group was disbanded, but its activities will nevertheless be continued by relevant stake holders, by a newly installed secretariat at *ZonMw* and by *FBG*. The government will remain involved, only more remote than in previous years.

Other aspects of the national strategy are to a large extent a continuation of already existing policies. Among these are the following:

- improving diagnosis and treatment of rare diseases,
- increasing the involvement of patient organisations in developing policies,
- screening for diseases,
- creating a specific scientific research programme,
- improving reimbursement for orphan drugs,
- registration of rare diseases and
- continued participation in European policies and policy development.

⁷ These resources are available through the subsidy of the past years.

⁸ This informational desk is not completely new. One of the tasks of the former Steering Committee on Orphan was to provide information to persons and others interested in the rare diseases and orphan drugs.

Diagnosis and treatment

редките заболявания (bg) zácna onemocnění (cz) sjældne sygdomme (dk)
seltene Krankheiten (de/be/at) haruldased haigused (ee) onáviēc vóooi (gr/cy)
maladies rares (fr/be/lu) rare diseases (uk/ie) malattie rare (it) retās slimības (lv)
retosios ligos (lt) ritka betegsések (hu) mard rari (mt) zeldzame ziektes (nl/be)
rzadkie choroby (pl) doenças raras (pt) bolile rare (ro) zriedkavé choroby (sk)
redke bolezni (si) enfermedades raras (es) harvinaiset sairaudet (fi)
sällsynta sjukdomar (se)

In patients with a rare disease, establishing a precise diagnosis and initiating a treatment are typically not straightforward and additional expertise is often required. Diagnosing a rare disease and treating the patient can, therefore, in many cases be considered as highly specialised medical care. The current government writes in its policy statement the following regarding specialised healthcare: *'The rarer, more complex or more innovative a treatment is, the greater the need to concentrate these treatments in a few top ranking hospitals....This will result in improved quality of top ranking healthcare.'*⁹ Following this statement, the Ministry convened with the Dutch Federation of University Medical Centres (NFU)¹⁰ Government funding for non-standardized care and medical research was already available to university medical centres.¹¹ In a letter to NFU regarding the Long Term Cycle of the Academic Component the Ministry indicated that the university medical centres should develop, in a more formal and coordinated manner than before, referral centres for patients with rare diseases.^{12,13} The financial resources that the ministry allocates to these medical centres via the Academic Component would then primarily be intended for the funding of care for patients with a rare disease (or to care for patients that would need specialized non-standardized care) and to the funding of research in these fields. It is clear that diagnosis, treatment and research in the area of rare diseases are often not reimbursed via the usual procedures. The Academic Component can therefore, provide reimbursement for diagnosis and treatment and funding for research. Reallocation within the Academic Component should be as follows: 80% is destined for diagnosis and treatment and 20% for research and innovation. Reimbursement of the more standardised top ranking specialist healthcare (as mentioned in the government's policy statement of September 2010) is consolidated through healthcare premiums. NFU also makes information available, via its website, for patients and health care providers, about diagnosis of and treatment options for rare diseases.¹⁴

One aspect is of particular importance. Reimbursement of orphan drugs administered within a hospital-care setting can only be claimed by designated centres of expertise (in practice these are the university medical centres).¹⁵ Currently, the Ministry does not foresee a more formal designation of centres of expertise for rare diseases, it rather favours a bottom up approach (by the university medical centres) instead of a top down approach (by the Ministry).¹⁶

⁹ Stated to Parliament by the Prime minister on 26 October 2010.

¹⁰ <http://www.nfu.nl/index.php?id=21>

¹¹ During the last years the funding was around € 80 million per centre. Besides spending the funding on care and research, no other (pre)conditions were set.

¹² Letter to NFU (12 July 2011)

¹³ Taking into account the prevalence upper limit (not more than five per 10 000 European citizens) as stated in Regulation (EC) 141 / 2000, it will be evident that the great majority of diseases in Europe fulfill this criterion and are in fact 'rare'. Choices, therefore, have to be made, as is implicit in the statement to Parliament, made on 26 October 2010.

¹⁴ NFU Portal Top Referente Functies.

¹⁵ The Dutch Healthcare Authority (*Nederlandse Zorgautoriteit*). Policy stipulation BR/CU-2045, "Performance and rates for specialist medical care", section 12.2: Add-ons – Expensive and Orphan drugs. One might could consider the reimbursement policy regarding university hospitals as a restricted way to designate expert centers in The Netherlands.

¹⁶ The exact number of rare diseases is not known, but estimates vary from 5000 to 8000. Knowledge and expertise are available with regard to relatively few rare diseases and even in these additional knowledge is needed. Although some guidance is necessary, as stated in the government's policy document (see reference 10)

Diagnosing a rare disease and treating the patient often requires specialised expertise. Such expertise is currently only limitedly available. A scheme is being devised for the treatment of Cystic Fibrosis (CF) involving interdisciplinary healthcare on a per patient basis and the funding thereof. This concept is currently being developed and will be tested in practice during the year 2012 in a pilot program at the Erasmus Medical Centre/Sophia Children's Hospital in Rotterdam. This project was commissioned by the patient support organisation for Cystic Fibrosis (the NCFS) and set up by medical specialists involved in the treatment and care of Cystic Fibrosis. The project involves a very close collaboration with various relevant parties including healthcare insurers.

Making information on hereditary diseases available to patients is another important aspect in the Ministry's strategy. It subsidizes with this aim the National Information Centre for Heredity, Fertility and Medical Biotechnology (*Erfocentrum*).¹⁷

and the Ministry's letter to NFU (see reference 13), a top down approach could also restrict the development of knowledge. Last but not least, the bottom up approach is supported by NFU.

¹⁷ The Erfocentrum is subsidized annually with an amount of € 250.000. From 1 January 2013, however, the standard annual subsidy will be replaced by subsidies for different projects. Details regarding these changes will be elaborated in 2012.

Involving patient support groups

Patient support groups, also known as patient organisations, are, as already stated, involved in the national strategy since this strategy was developed since 1995 and implemented since 2001. The umbrella organisations NPCF/ CG-Council and VSOP (the Association of Collaborating Parent and Patient Organisations) participated in the steering group and as such were able to contribute to the past and present national strategy. These parties in turn, maintain relations with the large group of patient support organisations that each focus on a certain rare disease or group of rare diseases. NPCF/CG-Council and VSOP will also participate in the feedback group supporting the new secretariat at ZonMw. Patient support groups will also have opportunities to participate in projects and make suggestions via the www.nationaalplan.nl, a website under the auspices of the new secretariat at ZonMw. Patients, furthermore, participate in the FBG, that before 2012 had set rare diseases and orphan drug occasionally on its agenda. In the new strategy rare diseases are also formally part of the remit of the FBG.

Patient support organisations are financially supported by the Ministry of Health, Welfare and Sport. The budget for subsidies is currently € 42 million per annum, but will be gradually decreased (within a period of three years) to € 23 million.¹⁸ Patient organisations have gained expertise as well and are thus in a position to apply this expertise to helping in to improving diagnosis, treatment and care of patients with a rare disease. The Ministry has separately granted a subsidy to VSOP.¹⁹ This subsidy concerns a collaborative project in which VSOP, in cooperation with 12 organisations, will develop and compile healthcare standards (Project: 'Healthcare standards for rare conditions: Patient in the spotlight'). The amount of the subsidy is slightly more than € 1.9 million; the project's duration will be from 2011 up to 2014.

The aims of the collaborative project is the improvement of the quality of care for rare conditions. The project is complimentary to a similar on-going collaborative effort at the VSOP which has its focus on 5 specific rare conditions. As of 2013, patient organisations will be able – based on the new policy framework for the subsidization of patient organizations and organisations for the handicapped – to receive subsidies for new initiatives to influence other parties based on their expertise²⁰.

¹⁸ As part of a general decrease in expenditures following the economic crisis.

¹⁹ Via the CIBG/PGO Fund – the implementation organisation for the Ministry.

²⁰ Parliamentary papers II 2010/11, 29 214, no. 60. This concerns subsidy provision #2: collective contribution to the effectiveness of healthcare, support and social participation. Projects can become eligible for subsidy if a minimum of 7 patient organisations are collectively (via their voucher) willing to contribute to the project.

Screening

The Netherlands has implemented, as many other countries have done, screening for a number of diseases. New-borns are screened for eighteen (18) diseases via a heel prick blood test. Screening takes place under the auspices of the National Institute for Public Health and the Environment (*RIVM*), a government agency. The 18 diseases that are screened for, are all rare. However, the rationale of the current policy is not to screen for rare diseases as such, but instead to determine whether health benefits can be obtained via a timely diagnosis and treatment. Screening for Cystic Fibrosis was included quite recently (1 May 2011.) The policy of The Netherlands with regard to screening will very likely remain unchanged for the first few years to come.

Youth healthcare is accountable for the follow-up of growth and development of children in The Netherlands. Local authorities (municipalities) must, based on the Public Health Act (*Wpg*), organise screening and follow-up of children. Essentially all children are screened and followed in line with professional guidelines. The aims of these procedures are the early detection of diseases and health risks. Rare diseases can, like the more prevalent diseases, thus be early detected.

Screening of hereditary tumours is implemented via the Foundation for the Detection of Hereditary Tumours (*Stichting Opsporing Erfelijke Tumoren -STOET*) this foundation registers families with a predisposition for hereditary malignant tumours. All hereditary tumours are rare. After establishing a diagnosis of a rare tumour in a patient, his relatives are screened via the out-patient clinic for hereditary tumours.²¹ Follow-up of these relatives is the remit of STOET. This organisation has its offices at Leiden University Medical Centre and is also subsidised by the Ministry.²²

²¹ Present in each of the eight university medical centres and the two cancer hospitals).

²²STOET is granted a subsidy of € 360.000 in 2012. However, from 2013- 2015 this subsidy will be gradually diminished. The aim of the Ministry is that in 2015 STOET will be part of a larger organisation.

Scientific research

In the section *Diagnosis and treatment*, it is stated that the Academic Hospitals/University Medical Centres receive annual funding within the framework of the Academic Component.²³ University hospitals can allocate 20% of this particular funding to innovation and research. The Ministry by itself a very restricted role in direct funding of scientific research. Instead, funding of basic and applied research (in the life sciences and other sciences) granted via the National Science Organisations (NWO and ZonMw). With regard to rare conditions and orphan drugs, the Ministry commissioned ZonMw to develop a scientific multi-annual programme with a budget of € 13.4 million. This programme, Priority Medicines for Rare Diseases and Orphan Drugs, started in 2011.

As of 1 January 2012 ZonMw launched the Proper Drug Use (GGG) programme. This programme is an integral research program with its focus on a proper application of medicinal drugs. The programme will also support research with regard to orphan drugs and drugs used in the treatment of rare diseases. Information about this theme and program is available via:

www.zonmw.nl/nl/themas/thema-detail/geneesmiddelen/thema-detail/.

Besides the national programme, The Netherlands financially contributes to the Framework Programmes of the EU via the multi-annual budget. Within the theme 'Health' in the consecutive Framework Programmes and the research program of the Directorate General for Health and Consumers of the European Commission, rare conditions and orphan drugs are a theme of increasing importance.²⁴

²³ University Medical Centers are each allocated, via the Ministry of Health, Welfare and Sport, annually extra funding in the order of € 80 million for expenditures that are not covered via the health insurance system.

²⁴ In the 7th Framework Program (2007-2013) of the EU, 313 million Euros has so far been allocated for 65 projects. The European Commission has reserved 108 million Euros for the next *call for proposals*.

Reimbursing orphan drugs

Särläkemedel (se) Weesgeneesmiddelen (nl/be) Medicamentos orfãos (pt)
Harvinaislääkkeet (fi) Arzneimittel für seltene Leiden (de/at/be)
Medicamentos huérfanos (ES) Medicinali orfani (IT) Orphan drugs (uk/ie)
Médicaments orphélins (fr) Ritka betegségek gyógyszerei (hu)
Leky pro vzácná onemocnění (cz) Leki sieroce (pl) Lieky siroty (sk)
Ritieji vaistiniai preparatai (lt) Lægemidler til sjældne sygdomme (dk) Zdravilo sirota (si)
Zāles reto slimību ārstēšanai (lv) Medicini orfni (mt) Лекарства сираци (bg)
Medicamente orfane (ro) Harva Kasutatavad ravimid (ee)
Afskipt lyf; lyf við sjaldgæfum sjúkdómum (is) Ορφανά φάρμακα (gr/cy)
Legemidler mot (no)

In The Netherlands, all orphan drugs authorised in the European Community, are available, either via the hospital setting or via the out-patient setting. Since 1 January 2012 the system for reimbursement inside hospitals was changed. Orphan drugs in the hospital setting are reimbursed via *add-on* funding. If a physician administers an orphan drug to a patient with a rare disease the hospital is entitled to make a declaration of expenses as an *add on* to reimbursement of the diagnosis and treatment of that specific rare disease.²⁵ However, only university medical centres are entitled to submit a charge of expenses for a particular orphan drug.

In the out-patient setting the system will be unchanged for the next two years. The Health Care Insurance Board (CVZ) assesses whether an authorised orphan drug can be included the basic health package. If so, orphan drugs are reimbursed, as most other authorised drugs, via the Medicines Reimbursement System (GVS). The following documents 'Affordable health care' and 'Conditional insurance package eligibility' further elaborate on this system.^{26,27} Pharmaceutical companies may submit an application for reimbursement via the GVS to the Minister of Health, Welfare and Sport. The procedure for application is available via a joint publication by the Health Care Insurance Board and the Ministry of Health, Welfare and Sports – "Procedure for the Evaluation of Extramural Medicines".²⁸

As of 1 January 2014, all orphan medicines will be reimbursed via the hospital setting. This implies that orphan drugs will be transferred from the Medicines Reimbursement System (GVS) to the hospital system for reimbursement. These changes will not have any negative effects on the accessibility to orphan drugs for patients with rare diseases. The new policy is part of a larger policy plan to transfer all specialist medicinal products to hospital financing and is therefore not limited to orphan drugs.²⁹ A

²⁵ However, the specific rare disease should be part of a Diagnosis/Treatment combination, a system via which hospitals submit expenses to health insurers. For specific information: Healthcare Authority (Nederlandse Zorgautoriteit): Beleidsregel BR/CU-2045, Prestaties en tarieven medisch specialistische zorg. (Policy stipulation BR/CU-2045, "Performance and rates for specialist medical care", section 12.2: Add-ons – Expensive and Orphan drugs)

²⁵ <http://www.rijksoverheid.nl/documenten-en-publicaties/kamerstukken/2011/03/15/kamerbrief-zorg-die-loont.html> Policy stipulation BR/CU-2045, "Performance and rates for specialist medical care".

²⁶ <http://www.rijksoverheid.nl/documenten-en-publicaties/kamerstukken/2011/03/15/kamerbrief-zorg-die-loont.html> (Dutch language only)

²⁷ <http://www.rijksoverheid.nl/documenten-en-publicaties/kamerstukken/2011/12/08/kamerbrief-over-voorwaardelijke-pakkettoelating.html> (Dutch language only)

²⁸ <http://www.cvz.nl/binaries/content/documents/cvzinternet/nl/documenten/rapporten/2011/rpt1103-beoordeling-extramurale-geneesmiddelen.pdf> (Dutch language)

²⁹ Scientific advancements/developments have resulted in specific medicines to become available to relatively small numbers of patients. These medications are generally quite expensive and the discrepancy in price compared with other medicines seems to be diminishing/ is no longer clear. In reality however, a completely different situation is going on. It concerns patients who have a relatively common disease (e.g. rheumatoid arthritis or breast cancer) but for whom specific medicines have been developed. These specific medicines are formulated for the treatment of a particular stage of the disease or because these patients have genetic characteristics which cause them to be unresponsive to the usual treatments (personalised medicines). These patients do not have a rare disease and the specific medicines they require therefore do not qualify as orphan drugs.

compassionate use programme is not foreseen. A physician who wants to treat a patient with an unauthorized medicinal product needs a permission from the Health Inspectorate. Moreover, unauthorised medicinal products (including orphan drugs) are not reimbursed.

Registration of rare conditions

The registration of patients with a rare disorder remains complicated. The Netherlands has collected some data in relation to rare diseases, but the scope of this information is far from optimal. According to information available to the Ministry, the situation with regard to registration is complex in other European Member States as well. EUCERD (the European Union Committee of Experts on Rare Diseases) currently analyses, in cooperation with the European Commission (Directorate General for Health and Consumers – DG SANCO), registration of patients with a rare disease. A European database for cancer is already in place for a number of years. Data of patients with rare tumours are anonymously recorded in this database, as well as data in relation to the effectiveness and safety of medicines (including orphan drugs). A similar database might be developed for patients with rare diseases.

Involvement in European policy formulation

The Netherlands is actively involved in working groups and formal advisory committees of the European Union such as the Committee for Orphan Medicinal Products and the Committee for specialist healthcare at the European Medicines Agency (EMA), the *Programme Committee of Experts* in the current 7th Framework Programme of the EU. The Netherlands is also committed to preparatory work with regard to Horizon 2020 that includes the 8th Framework Programme. Horizon 2020 will start in 2014. The Netherlands furthermore, has a seat in EUCERD, a committee at the Directorate General for Health and Consumers at the European Commission that was established to improve healthcare for patients with a rare disease.

ANNEX

A FEW GENERAL DATA WITH REGARD TO HEALTH CARE IN THE NETHERLANDS

Population: 16.750.000 Million (August 2012)

GDP (estimate for 2011): € 624 (Billion)

Per capita income (2011/IMF-ranking nr. 10)³⁰: \$ 50 355 / € 38 365 (exchange rate December 2011: 1.34)

Key concepts in Health Care:

Solidarity, equal access, cost containment without detriment to quality of care

Financing the collective system of health care via:

Social Premium System (90%), Tax System (10%)

Expenditures (estimates) 2011: € 64.40 (x 1.000.000.000)³¹

Hospital care, primary care, dentists, etc.....	36,17
Among which are: primary care physicians.....	2,3
and	
expenditures on medicinal products (hospital and primary care).....	5.5
Long term care (nursing homes etc.).....	24,65
Others.....	3,55
 Total.....	 64,40

Average (2011): € 3840 Euro per annum per citizen

Expenditures on orphan medicinal Products (2011): at least 260,80 Million (in 2007: 113,90 Million)³²

³⁰ List of countries by GDP (nominal) per capita – Wikipedia August 2012.

³¹ Zorgbalans – RIVM, 2012. Figures for 2011 (and 2010) are not yet definite.

³² GIP/College voor zorgverzekeringen & RIS / Nederlandse Zorgautoriteit. These expenditures relate to out-patient treatments and to 11 orphan drugs for in-hospital treatment, reimbursed until the end of 2011 via a specific procedure.