

EARLY ACCESS PROGRAMMES IN THE NETHERLANDS: CHALLENGES AND OPPORTUNITIES ACROSS THE PATIENT JOURNEY

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BACKGROUND

The Dutch Medicines Act (Geneesmiddelenwet) prohibits (art. 40) the sale, distribution or supply of any medicine that has not been granted a marketing authorization in the Netherlands. However, the Act also provides for a number of exceptions in specific circumstances. These include magistral preparation of medicinal products for individual patients, Compassionate Use Programs (CUPs), and the supply of unregistered medicines through a Doctor's Declaration (also referred to as a Named Patient Program, NPP). Magistral preparation and supply under a Doctor's Declaration are not limited to early access situations. These mechanisms may also be used outside the context of early access, provided the statutory conditions are met. All exceptions aim to address unmet medical needs in patients with life-threatening, long-lasting, or seriously debilitating diseases for which authorised medicinal products do not provide a satisfactory treatment option. CUPs are regulated under Article 83 of Regulation (EC) No 726/2004, and are intended for a group of patients. In the Netherlands, the MEB is the competent authority for approval of CUPs. Supply of medicines through a Doctor's Declaration is governed under Article 40, paragraph 3c of the Dutch Medicines Act and applies exclusively to individual patients. Prior permission from the Health and Youth Care Inspectorate (IGJ) is required before an unregistered medicinal product can be supplied under this route.

Following European marketing authorisation, a complex transition phase often arises in the Netherlands before national availability, access, and reimbursement are secured. As highlighted in several recent publications (1–3), important questions remain regarding the appropriate duration of CUPs and NPPs and whether new patients may continue to be enrolled after marketing authorisation. Additional uncertainties concern unequal access to early access programmes across hospitals and patient populations, how to proceed in cases of delayed reimbursement, non-reimbursed indications, or reimbursement granted under more restrictive criteria. It also remains unclear whether there is an obligation to continue supplying a medicinal product until reimbursement decisions are finalised.

Against this backdrop, the Regulatory Science Network Netherlands (RSNN) organized an expert meeting in Utrecht on 19 May 2026. The meeting focused on how existing early access programmes can be better aligned with clinical practice to ensure continuous and timely patient access to medicines during transitions between regulatory approval, marketing authorization, and reimbursement.

EXPERT MEETING

During the meeting, representatives from patient organisations, healthcare professionals, industry, regulatory authorities, and policymakers reflected on the key challenges and potential solutions related to early access programmes. Prior to the meeting, the following questions were shared with the participants:

- What practical challenges do you encounter in the use of existing early access programs (such as CUPs, Named Patient Programs) in daily practice?
- What concrete recommendations or policy adjustments could help overcome these challenges and better address unmet patient needs during the transition from regulatory approval to national availability and reimbursement?

Participants explored these issues from the perspective of their own expertise and the meeting was conducted under the Chatham House Rule. For the purpose of reporting, the challenges and potential solutions discussed were organised according to a patient journey consisting of eight consecutive phases (see Figure 1). The following sections provide a summary of the key findings. Although challenges were identified across all phases of the patient journey, nearly all participants identified the period between marketing authorisation and reimbursement as a bottleneck.

PATIENT JOURNEY – 8 STEPS

From unmet need to sustainable access



PURPOSE OF EARLY ACCESS PROGRAMS

To provide patients with serious conditions early access to potentially effective drugs when standard treatment options are not available.

Figure 1: Patient journey in 8 steps

OVERARCHING OBSERVATIONS

Visibility and Equitable Access

Access to early access programmes, particularly NPPs, largely depends on the knowledge, experience, and professional networks of healthcare providers and treatment centres. As a result, patients may have unequal opportunities to access innovative therapies.

Organisation and Harmonisation

Administrative burden, procedural differences, limited money, and limited capacity present significant challenges in the implementation of NPPs and CUPs for both programme sponsors and those responsible for their execution. Greater harmonisation and national coordination were identified as key areas for improvement.

Continuity of Access

Nearly all participants identified the period between marketing authorisation and reimbursement as one of the most significant bottlenecks, alongside getting access to a possible treatment that has not been granted access. Greater predictability and better alignment between regulatory approval, reimbursement, and patient access pathways are needed to ensure continuity of care.

Patient Involvement

Across all phases of the patient journey, participants emphasised the importance of actively involving patients in information provision, decision-making, and policy development.

STEP 1: UNMET MEDICAL NEED

For patients for whom no appropriate treatment options are (or are no longer) available through routine clinical care, and for whom no suitable clinical trials are available for participation, access to treatment may be possible through an early access programme.

During the discussion, it became apparent that the term early access is interpreted in different ways. For the purposes of this expert meeting, the discussion focused on programmes that provide access to medicinal products prior to routine marketing authorisation and reimbursement, including Compassionate Use Programs (CUPs) and Named Patient Programs (NPPs). Other access pathways, such as exemptions, supply under a Doctor's Declaration in the context of medicine shortages, or the off-label use of authorised medicinal products, were outside the scope of this meeting.

Participants emphasised that early access programmes can provide an important treatment opportunity for patients when no standard therapeutic options are available. At the same time, it was recognised that these programmes are not always sufficiently visible within the

healthcare system, meaning that not all patients who may be eligible are identified and able to access them.

STEP 2: IDENTIFICATION OF POSSIBILITIES

Participants noted that identifying opportunities for early access largely depends on the knowledge, experience, and engagement of individual healthcare professionals, patients and treatment centres. Not all hospitals have experience with CUPs and NPPs, and awareness of the available access pathways also varies among physicians. As a result, patients with similar medical needs may have unequal opportunities to access innovative treatments depending on where they receive care. Participants considered this form of postcode-dependent access to be undesirable.

Participants also observed that there is no central overview of available early access programmes. Industry explained that, under the current regulations governing the promotion of medicinal products, they are not permitted to proactively publicise such programmes. Consequently, healthcare professionals often rely on existing professional networks and personal contacts to remain informed about available opportunities.

Participants therefore emphasised the importance of improving access to information. Professional societies and centres of expertise could play an important role by sharing knowledge, experience, and information about ongoing early access programmes. In addition, patient organisations were recognised as valuable partners through their close collaboration with these stakeholders and their ability to disseminate relevant information to their members when programmes become available that may be of interest to their patient communities. At the same time, it was acknowledged that the current regulations governing the promotion of medicinal products apply to all stakeholders, meaning that neither pharmaceutical companies nor other organisations can proactively communicate about unregistered medicinal products or early access programmes.

STEP 3: DECISION-MAKING: NPP OR CUP?

Participants discussed the considerations involved in choosing between a NPP and a CUP. The discussion revealed differing views on when a CUP should be initiated. Some participants assumed that a CUP would only be considered once multiple NPP requests had been submitted for the same medicinal product. Representatives from industry clarified that this is not the case. The decision to establish a CUP rests with the pharmaceutical company and is not dependent on a predefined number of ongoing NPP requests. It was also explained that NPP requests may be initiated by either the treating physician or the company.

Participants further noted that companies do not always have a complete overview of the number of ongoing NPP requests. As a result, they may lack a comprehensive understanding of the actual demand for a particular medicinal product within the Netherlands. Consequently, the decision to establish a CUP is based on a range of considerations, including anticipated clinical demand, international considerations, ongoing or planned clinical trials, consultation with the Dutch Health and Youth Care Inspectorate (IGJ), and the possibility towards a positive reimbursement status.

It was also noted that companies can generally implement an NPP more quickly than a CUP. As a result, NPPs are often introduced earlier in the drug development process, whereas a CUP is more commonly considered once the demand for treatment becomes broader and more clinical experience with the medicinal product has been gained. Limited national coordination means that similar situations may be assessed differently, leading to variation in approaches across healthcare institutions and companies.

Several participants advocated for greater central coordination of processes and information exchange, for example through professional societies and companies, while recognising that such coordination must take place within the boundaries of the applicable regulatory framework governing communication about unregistered medicinal products. They also emphasised the importance of early dialogue between pharmaceutical companies, healthcare professionals, patient organisations, and relevant public authorities to jointly determine the most appropriate access pathway. Some participants further suggested that the Ministry of Health, Welfare and Sport (VWS) could play a role in defining clear conditions for temporary access arrangements during the reimbursement assessment and negotiation period, for example through shared-risk models.

STEP 4: SET-UP OF THE PROGRAM

Many participants described the programme set-up phase as administratively burdensome, particularly for CUPs. Differences between procedures for NPPs and CUPs, variation in national systems across Europe, and the use of different protocols by individual companies all contribute to this complexity. At the same time, participants highlighted the limited capacity within hospitals, hospital pharmacies, and smaller companies, placing additional pressure on the implementation of these programmes.

A recurring concern regarding the initiation of a CUP was the uncertainty surrounding the future reimbursement of a medicinal product and, consequently, the expected duration of the CUP. This was considered particularly relevant in the Netherlands, where companies may face uncertainty about whether reimbursement will ultimately be granted and how long the assessment and negotiation process may take. According to participants, this uncertainty may

influence companies' willingness to initiate a CUP, meaning that bottlenecks arising later in the patient journey already affect decisions made during the programme set-up phase. In addition to the absence of a maximum duration for CUPs and the lack of clarity regarding when they should be discontinued, continued access following programme completion was identified as a key concern. Both patients and industry expressed the need for greater clarity regarding the duration of programmes and the possibilities for continuing treatment.

To address these challenges, several potential solutions were discussed. Participants suggested exploring further harmonisation of processes between companies, hospitals, and pharmacies, as well as closer coordination between the CBG, IJG and VWS. They also highlighted the potential value of temporary bridging mechanisms to better ensure continuity of access. Experiences and existing approaches in countries such as Belgium, France, and Denmark were cited as potential sources of inspiration.

STEP 5: TREATMENT IN AN NPP/ CUP

During treatment within NPPs and CUPs, several practical challenges were identified. Participants noted that the identification of opportunities (as described in Phase 2), and consequently access to these programmes, varies between hospitals and treatment centres. Patients are therefore largely dependent on the experience, capacity, and willingness of individual centres to implement a programme. Participants noted that the visibility of NPPs and CUPs differs. While approved CUPs are publicly listed by the Dutch Medicines Evaluation Board (MEB), no comparable overview exists for NPPs.

Participants also emphasised that the implementation of NPPs and CUPs can place a considerable burden on both treating physicians and hospital pharmacists. Organising the supply of medicinal products, supporting patients, and fulfilling administrative requirements require substantial time and resources. Some participants noted that hospitals may become more reluctant to participate when such programmes place a sustained demand on limited staff capacity.

Data collection was another important topic of discussion. From various perspectives, participants recognised that data generated through NPPs and CUPs can provide valuable insights, particularly for rare diseases and small patient populations where clinical evidence is limited. At the same time, they highlighted the practical and legal challenges associated with collecting data within these programmes. The opportunities for data collection differ between NPPs and CUPs and are partly determined by national and international regulatory frameworks.

Several participants emphasised the need for greater clarity regarding which data may be collected, under what conditions data collection is permitted, and how these data can contribute to further knowledge generation. It was also noted that patients should be actively involved in determining which outcome measures are most relevant when data collection forms part of a programme.

Potential solutions discussed included the development of national protocols, greater harmonisation between centres, and clear frameworks for data collection. These measures could improve both the feasibility of programme implementation and the usefulness of the data collected.

STEP 6: REGISTRATION & EUROPEAN APPROVAL OF THE DRUG

Participants regarded the positive opinion issued by the European Medicines Agency (EMA), followed by approval by the European Commission, as important milestones in the development of a medicinal product. At the same time, they emphasised that marketing authorisation does not automatically mean that patients gain immediate access to the medicinal product through routine clinical care.

At the time of marketing authorisation, questions arise regarding the future of ongoing NPPs and CUPs, the conditions under which new patients may be included, and how the transition to routine access of a medicinal product should be managed. From both the patient and healthcare professional perspectives, it was stressed that clear communication at the start of programs about this transition, is essential. Patients want timely information about the possible next steps following marketing authorisation, while healthcare professionals require clarity regarding the available access pathways for patients who are already receiving treatment.

Participants therefore emphasised the importance of early agreements on the transition from early access to the reimbursement phase. Clear communication with patients and healthcare professionals regarding possible next steps, programme discontinuation, and alternative access pathways was considered a key prerequisite for ensuring a smooth transition following marketing authorisation.

STEP 7: TRANSITION TO REIMBURSEMENT

Nearly all participants identified the period between marketing authorisation and reimbursement as the greatest bottleneck in the patient journey alongside being to participate in an early access program. Following marketing authorisation, it may take considerable time before a medicinal product becomes available through the routine

reimbursement system. This creates a period of uncertainty for patients, healthcare professionals, and industry.

From the patient perspective, participants emphasised that uncertainty regarding continuity of treatment has a significant impact. Patients want to know whether they will be able to continue accessing a medicinal product once an NPP or CUP ends, and what alternatives are available if reimbursement is delayed or not granted. Participants noted that negative experiences with poorly organised follow-on access may undermine trust in both clinical research and early access programmes.

Healthcare professionals also face dilemmas during this phase. When a pharmaceutical company decides to discontinue a programme, or when reimbursement takes longer than anticipated, physicians may be confronted with difficult ethical and legal decisions regarding whether to continue or discontinue treatment.

Representatives from industry indicated that uncertainty surrounding reimbursement, the duration of national procedures, and the lack of predictability are important factors in the decision to establish a CUP. As medicinal products provided through a CUP are generally supplied without reimbursement, there is limited willingness to continue such programmes for extended periods when it is unclear whether, or when, reimbursement will be granted. Several participants noted that this uncertainty may discourage companies from initiating a CUP, particularly in the Netherlands.

Several forms of temporary or conditional access were discussed as potential solutions. Participants referred to bridging arrangements and early access models in countries such as Belgium, France, and Denmark. Drug Access Protocol (DAP)- and Orphan Drug Access Protocol (ODAP)-like mechanisms (4,5) were also mentioned as examples of approaches that may provide inspiration for bridging the period between marketing authorisation and reimbursement. Participants emphasised, however, that these programmes operate in a different regulatory context. DAPs and ODAPs generally apply to medicinal products that have already received marketing authorisation for one or more indications but are being used or investigated for another indication. In contrast, NPPs and CUPs generally concern medicinal products that have not yet received marketing authorisation. Although the underlying regulatory frameworks differ, participants recognised a similar need in both situations for temporary access during the transition period.

Another proposal was to establish a clearer distinction between the periods before and after marketing authorisation. Under such an approach, access prior to marketing authorisation through NPPs and CUPs could continue under arrangements agreed between the pharmaceutical company and the hospital pharmacy, while a form of temporary or

conditional reimbursement could be considered after marketing authorisation until a final reimbursement decision has been made. According to several participants, such an approach could contribute to greater continuity of care for patients and increased predictability for both healthcare professionals and companies.

Finally, participants suggested that the collection of relevant data during this phase could provide an additional incentive for companies to initiate and continue early access programmes.

FASE 8: ROUTINE ACCESS & REIMBURSEMENT

Participants emphasised that the ultimate goal of early access programmes is not only to provide patients with early access to innovative treatments, but also to facilitate a smooth transition to routine access of a medicinal product. At the same time, it was recognised that this transition is not always seamless in practice.

Several participants noted that patients who have been successfully treated within an NPP or CUP may once again face uncertainty when a medicinal product becomes part of routine clinical care. This may, for example, result from differences in reimbursement, implementation, or the definition of the patient populations eligible for treatment.

Participants also observed that the experiences from the earlier phases of the patient journey illustrate how closely marketing authorisation, reimbursement, and patient access are interconnected. When these processes are insufficiently aligned, delays and inequities in access between patient groups may arise.

There was broad consensus among participants that better alignment between regulatory, reimbursement, and access pathways is essential to ensure continuity of care. Participants also advocated for a more structural integration of early access into the Dutch healthcare system, enabling patients with an unmet medical need not only to obtain timely access to innovative treatments, but also to retain access once a medicinal product becomes available through routine clinical care.

CONCLUSION

The expert meeting highlighted that early access programmes are an important contribution in providing timely access to innovative medicinal products for patients with an unmet medical need. At the same time, significant challenges remain with respect to harmonisation, coordination, and continuity of access.

The challenges described in this report primarily concern access to medicinal products before marketing authorisation. For certain patient groups, including children, additional access challenges may arise after initial marketing authorisation, as paediatric authorisation, reimbursement, indication extensions, and the availability of age-appropriate formulations often become available at a later stage. These issues were recognised but were outside the scope of the present expert meeting.

Participants expressed a shared commitment to continue working collaboratively on solutions that improve the alignment between regulation, marketing authorisation, reimbursement, and patient access. They emphasised that an integrated approach encompassing the entire patient journey is essential to fully realise the potential of early access programmes.

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