# Named Patient Programs
## Provide Pre-Launch Access to Drugs

**The dire outlook facing AML patients motivated a company to provide its drug in advance of the commercial launch.**

Contributed by

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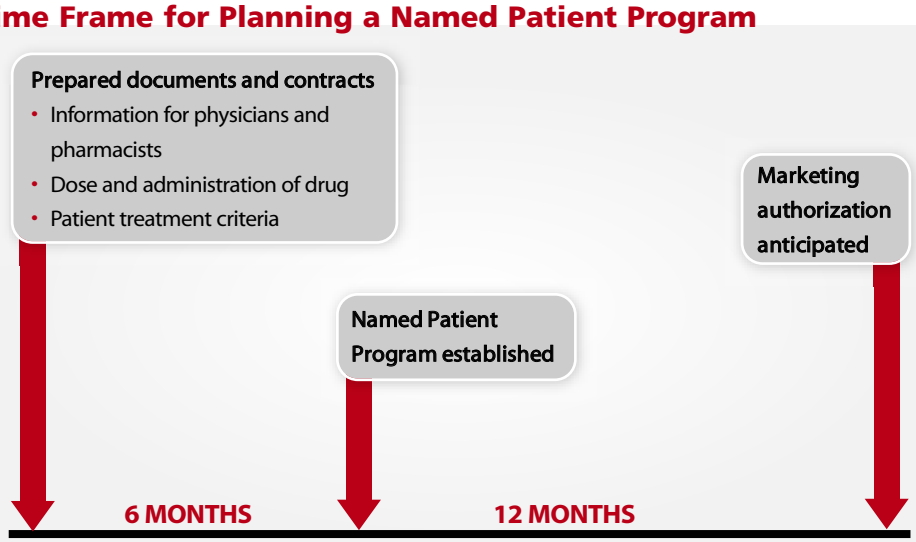
EpiCept is a specialty pharmaceutical company that focuses on fulfilling unmet medical needs in cancer treatment and pain management. The company possesses an approved cancer product and a deep and balanced pipeline of three major clinical product candidates. For more information, visit epicept.com or e-mail jtalley@epicept.com.

***The grim prospects for long-term survival in acute myeloid leukemia (AML) strongly motivated EpiCept to provide patients a route of access in advance of the commercial introduction of its drug, Ceplene, the first approved immunotherapy for remission maintenance and prevention of relapse in adult AML patients in first remission.***

### Time Frame for Planning a Named Patient Program




Note: The optimal time frame for implementing the program is about 12 months before the drug is expected to receive approval.

Named patient programs (NPPs) enable physicians and patients in Europe to access medications that have been approved by the European Medicines Agency (EMEA), but not yet launched commercially in European Union (EU) member countries. Regulations also allow patients to access drugs that are approved outside of the EU, but not yet in their home countries. In both scenarios, requests for access are made by physicians on behalf of individuals, or named patients.

Ceplene is available on a named patient basis in Europe and in dozens of other key markets, including Latin America, Asia/Pacific, Australia, Israel, and Canada.

Similar programs that enable pre-approval or pre-launch access exist in many countries around the world; respective governments have set well-defined rules for access criteria, collection of safety data, and control of drug distribution. In the United States, such government-sanctioned initiatives are referred to as expanded access programs and may be implemented for individuals, intermediate-sized groups (10 to 100 patients), and large groups of patients.

### THE ROUTE TO ACCESS

At the heart of the decision to establish a pre-launch or pre-approval access program is the patient with an unmet medical need. EpiCept sought to offer a route to access that was legal and ethical. Fueling interest in providing access were numerous requests being received by the company from physicians in Europe and other parts of the world.

The successful development and implementation of NPPs require the involvement and coordination of many disciplines within the sponsoring company, including representatives from medical affairs, pharmaceutical development, regulatory, supply chain, business development, and finance. The cross-functional team is responsible for ensuring that the clinical criteria for patient participation are established, physician educational materials are defined, the supply of drug is adequate to support the program, and enrollment in any ongoing clinical trials will not be adversely affected.

The NPP has allowed EpiCept to make Ceplene available to patients in the following situations:

- In the EU following centralized approval but prior to commercial launch in member countries.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## Named Patient Program Requirements in Select EU Member States

| Country | Common Name of Program | Type | Limited to Products Licensed Elsewhere? | Authorization Necessary? | Application to Competent Authority? | Applicant | Prescription Required? | Liability | Who Pays? | Follow-up Obligations |
|---|---|---|---|---|---|---|---|---|---|---|
| **France** | Nominative temporary authorization for use | Named | Not required | Yes | To French Medicines Agency (AFSSAPS) | Physician | Yes | Company (quality) Physician (clinical use) | Hospital, national insurance system | AE reporting managed by AFSSAPS |
| **France** | Cohort temporary authorization for use | Cohort | Not required | Yes | To French Medicines Agency (AFSSAPS) | Company | No | Company | Hospital, national insurance system | AE reporting managed by company |
| **Germany** | Compassionate use | Named | Required | No | No | Varies | Yes | Company | Dealer or patient | AE reporting as in clinical trials |
| **Italy** | Therapeutic use of drug undergoing clinical trial | Named | Not required | No | To local ethics committee | Physician or group of physicians | No | Physician | Patient | No specific rules; expected to be the same as for other medicines |

- In countries where applications for approval were being prepared.
- In countries where approval won't be sought.

While the drug approval process in Europe is centralized via the EMEA, the drug reimbursement system is decentralized; decisions regarding reimbursement take place within each respective EU country. This process often results in a delay in commercial launch as some countries can take between 12 and 18 months to establish reimbursement following EMEA approval of a drug. During this lag period, AML patients in the EU can access Ceplene through the NPP.

While pre-launch access is permitted by EU legislation, NPPs are governed by the individual member states; each of the 30 member states of the European Economic Area (EEA) has its own regulations regarding access to pre-launch medicines.

To navigate the regulatory and logistical pathways in more than 100 countries and remain in compliance with all authorities, EpiCept chose to partner with Idis, a specialist in NPP development and management, rather than rely on internal resources.

Once the decision was made to offer Ceplene on a named patient basis, establishment of the program required about 10 weeks. Initiation of the NPP in the EU was timed to coincide with the date the drug was ready from a manufacturing standpoint and had complete and final EMEA approval and sign off.

Along with each request for Ceplene the prescribing physician provides the patient's age and remission status. EpiCept provides complete prescribing information as contained within the Summary of Product Characteristics (SmPC) to those physicians requesting the drug. A copy of a peer-reviewed article on Ceplene's Phase III clinical trial is also provided.

As with all NPPs, the sponsoring company has a responsibility to report serious adverse reactions to the local regulatory authorities.

Working with Idis, EpiCept established a full pharmacovigilance system to capture and report such information. The company also established a series of standard operating procedures to guide the program — these include procedures related to enrollment of patients, release of the drug to the hospital, as well as regular reporting mechanisms on usage.

Programs in the various countries will terminate with the commercial launch of the drug. Those patients accessing Ceplene on a named patient basis will simply transition to the commercial drug once available and continue treatment in an uninterrupted manner. In countries where approval is not being sought, patients will continue to access Ceplene on a named patient basis.

## MEETING UNMET NEEDS

Pre-launch access to drugs can provide profound benefits for patients with unmet medical needs when all other therapeutic options have failed or no other options are available. NPPs facilitate patient access in markets that a company may not be pursuing in any other way. For example, it can be impractical for a small company to credibly pursue approval in more than 100 countries, such as those that the Ceplene NPP encompasses.

Using NPPs, access can be expanded to markets that would otherwise not be served, where gaps exist between approval and launch or in situations where a patient cannot enter a clinical trial.

Named patient programs can provide access at a number of stages throughout a product's life cycle:

- During Phase III, for patients with an unmet medical need who do not meet a clinical trial's inclusion criteria.
- Bridging the gap between the end of Phase III and receipt of marketing authorization.
- Bridging the gap between approval and commercial launch.
- Throughout a staggered global launch while approval and reimbursement is being sought across countries worldwide.

For patients with life-threatening illnesses such as AML, license approval or commercial launch of an innovative new drug in their home country may come too late. In some cases — those involving the EU registration and approval process, for example — a drug may not clear reimbursement hurdles in an individual country until well after centralized approval is granted. In cases where a formal launch may not even be planned, a patient may not have any other opportunity to receive the drug other than through a named patient program.

These programs also provide access to patients who would otherwise not be able to participate in company-sponsored clinical trials and allow sponsoring companies to engage with physicians and provide early, hands-on access to life-saving medicines. In all of these scenarios, named patient programs allow companies to provide access to innovative drugs in an ethical, legal manner while maintaining strict control over where the drugs go and how they are used. ✦

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