

Implementing reference frameworks to enable early access to innovative therapies



TYPE
STATUS

Guidance for implementation

LAST
UPDATE

June 2021

EU COUNTRIES
Diagnostic and treatment - early access to innovative therapies

PROBLEM & OBJECTIVE

Because all countries in Europe don't have framework to enable early access program to innovative immunotherapies, the iPAAC WP9 aimed to identify and characterize existing programs or reference frameworks enabling an early access to innovative immunotherapies for unauthorized indications in order to have an overview of the panel which could help countries to develop or improve their own solution. The WP9 also analyzed the opinions and expectations from relevant stakeholders regarding access to innovative immunotherapies (such as checkpoint inhibitors, CAR-T cells) to highlight strengths of these programs as well as potential for improvement.

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KEY COMPONENTS / STEPS

- The following methodological steps were followed:
 - A literature review, and the dissemination of a questionnaire among iPAAC partners in Q4 2018, were conducted to identify and characterize existing early access programs in Europe.
 - Opinions from stakeholders regarding access to innovative therapies were collected via another dedicated questionnaire.
- Concerning the steps to set up such early access program, the study carried out did not question these aspects.

KEY CONTEXTUAL FACTORS

- Replies to the first questionnaire were collected from 23 different countries. About half of the countries (10/22, 45%) had an existing program enabling early access to innovation cancer therapies (before marketing authorization or before extension of indication). This includes compassionate use programs with financial support provided by pharmaceutical industries; but some countries have other kind of programs in place with public financing, such as France, Portugal and Germany. Opinion on access to innovative immunotherapies was collected from 54 stakeholders including healthcare professionals, HTA agencies, health & medicines agencies, cancer institutes and patients, from 20 different countries. Less than half of the repliers (47%) were able to say that there was such an existing program enabling the access to innovative immunotherapies prior marketing authorization in place in their country. However, the satisfaction regarding implemented programs was high: among the 24 positive replies mentioning that there was an existing early access program in place in their country, 80% were satisfied with the implemented system.

MAIN IMPACTS / ADDED VALUE

- Frameworks enabling early access to innovative therapies were overall seen as a good help to improve access to new treatment. Implementation of early access programs can indeed help bridging the gap between EMA approval and definition of the price as well as decision for reimbursement in member states.
- The list and description of existing early access programs in Europe is available in the WP9 deliverables: Reference frameworks linked with the access to innovative immunotherapies.

LESSONS LEARNED

- Two main aspects stood out for an efficient implementation of early access programs to innovative cancer therapies:
 - Having clear defined pathways and legal frameworks. It appears for instance very important to define in advance:
 - clear procedure to enable an adequate monitoring of benefits and risks of the innovative treatment ;
 - centers where the treatment could be received;
 - funding systems for patients on treatment, including in the event of a negative reimbursement decision.
 - Strengthening dialogues and collaborations among the different stakeholders involved in these programs:
 - strong discussion between payers, prescribers and other relevant stakeholders should occur all along the implementation of such programs;
 - provide relevant information to patients regarding availability of innovative therapies is also very important. Patients should be clearly informed if they are receiving a drug with no approved marketing authorization.
- Furthermore, the system implemented should make sure to avoid any potential inequalities between healthcare centers and regions.

REFERENCES & DOCUMENTATION

- iPAAC WP9 task 1 deliverable: Reference frameworks linked with the access to innovative immunotherapies.

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