EMA

Involvement of patient representatives in scientific advice procedures at EMA

What is scientific advice (SA)?

Scientific advice is when a medicine developer requests advice from the European Medicines Agency (EMA) for a medicine they are developing. This advice may be requested at any stage or on any aspect of development of a medicine from:

- manufacturing,
- non-clinical,
- clinical (appropriateness of studies in patients or healthy volunteers, selection of endpoints, post-authorisation activities including risk-management plans) or
- methodological issues.

The advice is based on documentation provided by the medicine developer with the objective of producing high-quality, effective and acceptably safe medicines.

Scientific advice can be given either in writing or in a face-to-face meeting (patients can choose to respond in writing or join via telephone).

The role of patient representatives

Patients are invited to share their real-life perspective and experience in relation to a particular medicine in their disease area. This can help medicines developers and regulators understand better what that patient group considers to be important.

Patients may be asked to contribute by participating in meetings with EMA and the medicine developer or by providing written feedback on the proposed development plan. Please note that all documents and meetings are in English and that EMA covers costs of travel and accommodation.

Outcome of patient participation in scientific advice

It has been shown that in nearly every case (93%) patient input provided added value to the scientific advice. In some cases patient input was critical in providing a fresh perspective and real life experience that resulted in a more practical and better designed development plan for the medicine.

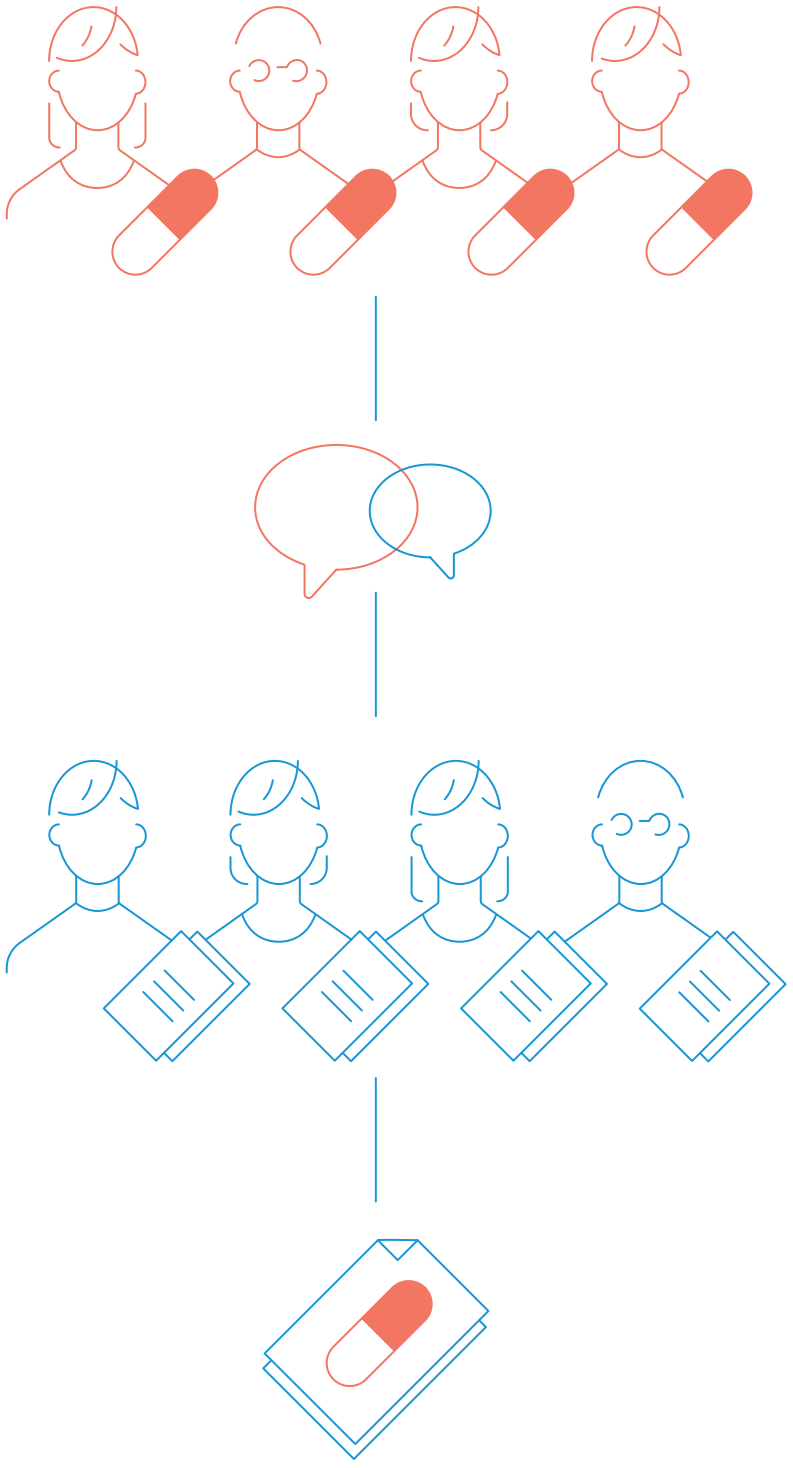
In over a quarter of instances where advice was given to medicines developers, this advice was modified to reflect patient input. In more than half the cases, patients' comments led to further reflection by regulators on the development plan.

This shows that patients can genuinely make a difference and help to develop medicines more efficiently, ensuring that effective, safe medicines reach those who need them as quickly as possible.

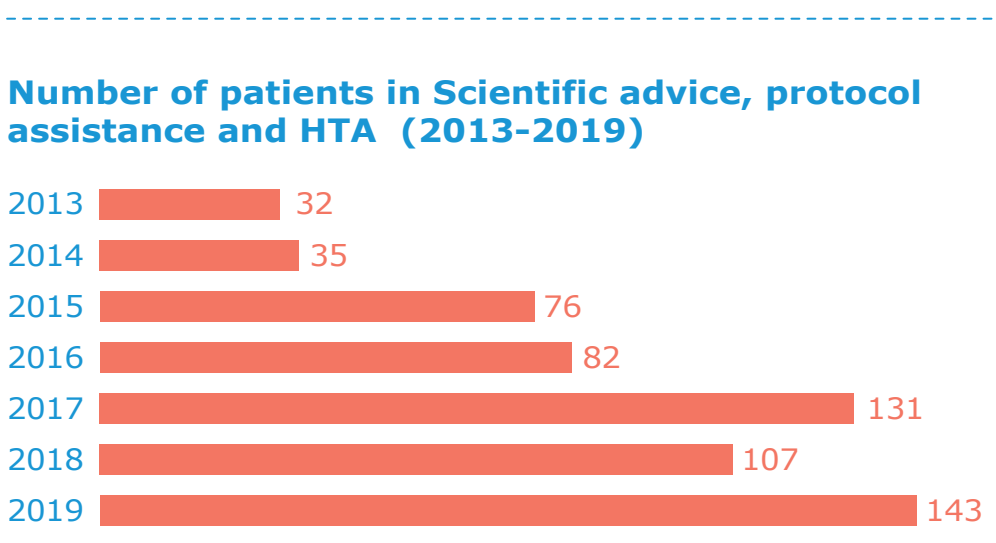
Who provides scientific advice?

Scientific advice is given by answering specific questions posed by a medicine developer. The Committee for Medicinal Products for Human Use (CHMP) is responsible for this advice, which is based on recommendations from the Scientific Advice Working Party (SAWP).

Patients have participated in scientific advice procedures since 2007 and their input has been shown to contribute to the improvement of the advice provided as well as increasing transparency and trust in the regulatory processes.



Number of patients in Scientific advice, protocol assistance and HTA (2013-2019)



What is the process?

Patient

Like all experts, patients are required to sign a confidentiality undertaking / declaration of interest

Read documents (Focus on clinical questions)

Contact EMA staff (if needed)

Send comments in writing (Day 40) or participate in a meeting (Day 60)

EMA

Patients are contacted by EMA or through an EMA eligible patient organisation.

EMA then sends information [called a 'briefing package'] on the medicine and its development plan. Patients are recommended to focus their input on the introduction and clinical questions and are not expected to be medical or scientific experts.

Support is provided by EMA throughout the process. Patients will be contacted by EMA staff responsible for the procedure and the process of scientific advice will be explained as well as where their specific input would be valuable.

If EMA decides to respond to the medicine developer in writing, patients are asked to provide comments (Day 40).

If EMA decides to meet the medicine developer the patient will be invited to attend (in person or via conference call) at Day 60. Travel and accommodation is organised and covered by EMA.

After participating, patients are sent a copy of the advice letter sent to the medicine developer, minutes (where a meeting has taken place) and a link to a questionnaire for feedback on their overall experience.

Patient input resulted in modification of advice to company — 27%

Patient input resulted in further reflection by coordinators —53%

In 93% of cases, patient input was considered to be of added-value