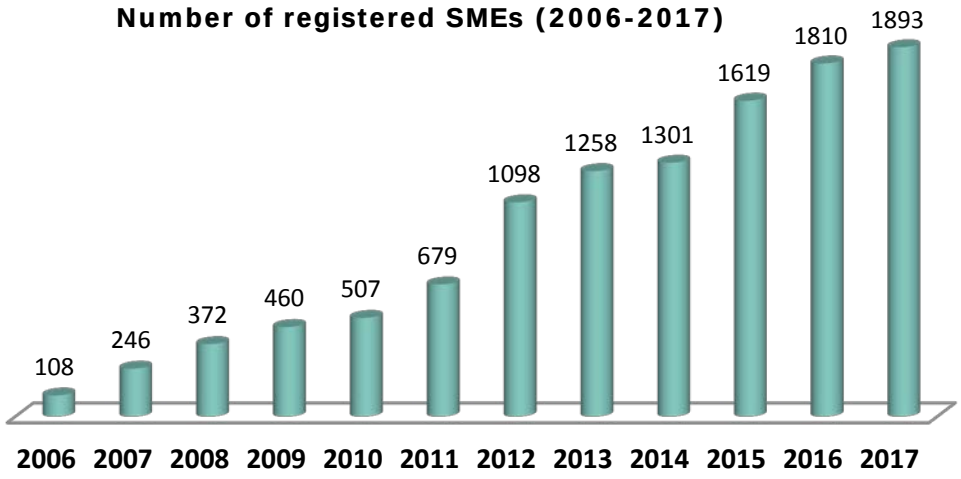
10 April 2018
EMA/123438/2018
Stakeholders and Communication Division

SME Office annual report 2017

The annual report is prepared by the EMA's SME Office and provides an overview of SME related activities at the European Medicines Agency in 2017.

1. Profile of registered SMEs

A total of 1893 SMEs were registered at year end 2017[1], which represents an increase of 5% compared to 2016.



The profile of registered SMEs in 2017 remained broadly similar compared to 2016:

- **Size**

Most SMEs (40%) were micro- (<10 staff; turnover or balance sheet < € 2 mil), 35% small- (<50 staff; turnover or balance sheet < € 10 mil), and 25% medium-sized companies (<250 staff; turnover < € 50 mil or balance sheet < € 43 mil).

[1] The data presented is based on the information provided in the SME declarations submitted to EMA and relate to companies with a valid SME status at year end 2017 (see online SME register).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5545
Send a question via our website www.ema.europa.eu/contact

- **Ownership and geographic distribution**

The majority of companies are privately owned by individuals (50%) or private corporations (20%). Funding by venture capital companies and other private investments accounts each for 8% and business angels for 6%. Nine percent of registered companies are academic spin-offs.

The highest proportion of SMEs are based in the United Kingdom (17%), Germany (13%) and France (9%) followed by Italy (6%) and Spain (5%).

Of all registered companies, 11% were incorporated over the last three years, with 43% of these being new subsidiaries of a parent company.

- **Areas of activity**

The large majority of companies are (bio)pharmaceutical companies developing or marketing medicinal products for human use (78%), 4% veterinary medicines, 4% products for both human and veterinary use and 14% are service providers to the pharmaceutical industry (e.g. regulatory consultancies, contract research organisations). About 20% of SMEs perform their activities in the medical devices field or have combined devices and medicines pipelines.

With regards to product pipelines, 12% of SMEs develop or market biologicals, 7% advanced therapies medicinal products, 22% medicines for orphan diseases and 10% paediatric medicines. Twenty three percent of companies develop or market generics.

2. Support to SMEs

2.1. Regulatory assistance

Requests for regulatory assistance from SMEs increased by 6% in 2017 (184). Assistance was provided through the SME Office helpdesk, email correspondence, teleconferences or meetings ('SME briefing meetings').

SME briefing meetings provide a platform for assistance to enterprises which are unfamiliar with the EU regulatory approval system and facilitate early interaction with the Agency. They provide an opportunity for SMEs to informally discuss a planned regulatory strategy and development programme.

In 2017, the SME Office set up 17 SME briefing meetings (12 face-to-face meetings and 5 teleconferences), which represents an increase of 31% compared to 2016.

SME briefing meetings covered different therapeutic areas and the majority were in the field of oncology for products in phase I/II of clinical trials.

Most meetings were multidisciplinary. Topics frequently discussed related to how and when to seek scientific advice to clarify legislative requirements, regulatory issues (e.g. legal basis for submission of marketing authorisation dossiers, conditional marketing authorisations, authorisations under exceptional circumstances), paediatric requirements, orphan designation and eligibility criteria for the PRIME scheme.

2.2. Scientific advice

For human medicines, 194 scientific advice and protocol assistance[2] were submitted by SMEs in 2017, which represents an increase of 10% compared to 2016.

[2] Protocol assistance is scientific advice for designated orphan medicinal products.

A quarter of all scientific advice (24%; 115/471) and half of all protocol assistance (50%; 79/159) were submitted by SMEs. Of those, 3 out of 29 (10%) requests for parallel scientific advice with health technology assessment (HTA) bodies and 7 out of 19 (37%) biomarker qualification requests were from SMEs.

As in previous years, most advice was sought on clinical development, in particular for confirmatory studies of anti-neoplastic and immunomodulating agents (28%). The highest proportion of scientific advice was for chemical entities (57%) followed by biologicals (24%) and advanced therapies medicinal products (19%). Out of 28 scientific advice requests for products eligible to the PRIME scheme (see below), 16 (57%) were from SMEs.

The number of scientific advice requests for veterinary medicines submitted by SMEs was 7 in 2017, which accounted for 35% of all requests (7/20). This figure is lower than in 2016 where 50% of scientific advice requests were submitted by SMEs.

2.3. Advanced Therapies Medicinal Products (ATMPs)

The figure for certification procedure for SMEs developing advanced therapies was the same as in 2016 (2).

2.4. PRIME scheme

Out of 81 PRIME applications received in 2017, half of them (40) were from SMEs and 4 submitted at the early entry stage (‘proof of principle’).

Out of all products granted PRIME eligibility in 2017 (19 products), 8 were from SMEs (3 ATMPs, 2 biologicals and 3 chemical entities). One was at the early entry stage.

Five products granted eligibility were for orphan diseases and the therapeutic areas most represented were oncology and infectious diseases.

3. Marketing authorisations

Outcomes presented below do not take into account products or SMEs acquisitions by larger companies prior to or during filing.

3.1. Human Medicines

From December 2005 to December 2017, out of 128 marketing authorisation applications (MAAs) which had a CHMP outcome, 71 received a positive opinion and 57 resulted in negative outcomes (13 negative opinions and 44 withdrawals).

In 2017, 22 % of all MAAs (20 out of 90) for human medicinal products were submitted by SMEs. The increased number of marketing authorisations applications submitted by SMEs in 2016 translated into high figures of marketing authorisation applications with an outcome in 2017, and the highest number of positive opinions to date. There were 12 positive opinions and 9 negative outcomes (2 negative opinions and 7 withdrawals), which represents a higher success rate (57%) than 2016 (40%).

3.2. Veterinary Medicines

From December 2005 to December 2017, out of 28 MAAs with a CVMP outcome, 23 received a positive opinion and 5 resulted in negative outcomes (1 negative opinion and 4 withdrawals). In 2017, 35 % of all MAAs were submitted by SMEs (6 out of 17). There were 2 positive opinions and no negative outcomes.

4. Engagement with stakeholders

The SME Office continued its outreach activities to SMEs to increase regulatory awareness and training.

Two SME Info Days were organised in 2017. The first event covered the new clinical trial regulation (Link). The second focused on EU initiatives to foster innovative medicines' development and early access (Link). It also engaged SMEs with the EU Innovation Network[3] and provided an update on Brexit related activities[4]. More than 200 participants attended each event (in-person or via broadcast).

Quarterly SMEs newsletters and targeted mailings provided updates on regulatory and scientific guidance or public consultations e.g. related to Brexit preparedness, Innovative Medicines Initiative and Horizon 2020 calls.

EMA also interacted with SMEs stakeholders and academia on a series of topics such as clinical data publication, Eudravigilance, ATMPs and parallel scientific advice with health technology assessment (HTA) bodies.

The SME office continued its interactions with DG GROW and EU Agencies' SME support structures on the revision of the SME definition and updated SMEs.

EMA launched in May 2017 an action plan to support SMEs[5]. It outlines a series of actions, identified in the 10-year report on the SME initiative, where support and engagement is considered important to advancing EMA's mission for SMEs over 2017-2020. The plan includes actions to increase awareness of EMA's SME initiative, by enhancing engagement with actors in the pharmaceutical innovation environment such as incubators, universities and investors. It also includes actions relating to training and education, support to innovative medicines' developments and engagement with SMEs, EU partners and stakeholders.

5. Conclusion

2017 saw an increase in the number of SMEs registered with the Agency and an increased uptake of scientific advice and protocol assistance, the PRIME scheme and regulatory assistance provided by the SME Office. The number of positive opinions following marketing authorisation applications were the highest to date.

The SME Office will continue in 2018 to inform companies developing or marketing human and veterinary medicines of regulatory actions that need to be taken, before the UK leaves the EU, to maintain SME status and allow the continuous marketing and supply of medicines within the EU.

EMA remains committed to fostering an environment supportive to SMEs and their stakeholders, as it implements the action plans for small and medium-sized enterprises, advanced therapies medicines[6]

[3] EU Innovation Network
[4] United Kingdom's withdrawal from the European Union ('Brexit')
[5] EMA action plan for small and medium-sized enterprises (SMEs)
[6] EC/EMA action plan on advanced therapy medicines

and academia[7] adopted in 2017, within the frameworks for interactions with industry stakeholders and academia.

Contacts for further information

E-mail queries: sme@ema.europa.eu

SME Office: +44 (0)20 3660 8787

[7] Action plan framework for collaboration between EMA and academia