EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Overview of the experience of Article 83 compassionate use opinions at EMA

Presentation for Expert Group on Safe and Timely Access to Medicines for Patients (STAMP)

Human Medicines Research and Development Support Division

Agenda

- ✓ Background
- ✓ Analysis of experience

Background (1/2)

- Article 83 of Regulation (EC) No 726/2004 introduced legal framework for Member State to ask the CHMP when **compassionate use for group of patients** is envisaged to adopt opinions on the conditions for use, conditions for distribution and the patients targeted

- Article 83 of Regulation (EC) No 726/2004 further states that when a Member State makes use of the possibility for compassionate use for group of patients it shall notify the Agency

- As part of the Commission expert group on 'Safe and Timely Access to Medicines for Patients' (STAMP), experience with CU at national level was discussed to identify ways to optimise the use of existing regulatory tools to further improve safe and timely access of medicines for patients

Background (2/2)

For the STAMP meeting on 10 Mar 2016, the EC asked the EMA to present

- Analysis of experience with CU at EU level

✓CHMP was also invited to share its views on this topic and identify questions for consideration by the STAMP

Analysis of experience

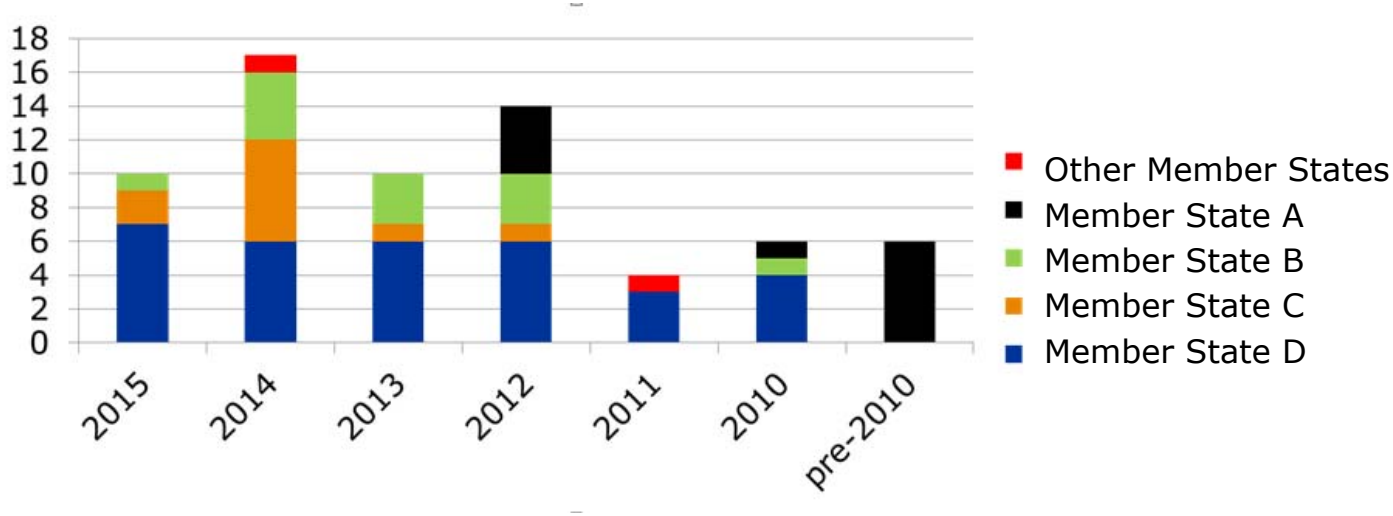
- Since the introduction of Article 83 of Regulation EC No 726/2004 in 2005, the CHMP adopted 5 scientific opinions for Compassionate Use for two conditions (hepatitis C and influenza)
- An analysis of the experience to date for **Compassionate Use intended for group of patients** at EU level demonstrates that few member states appear to follow the requirements to notify the EMA about nationally implemented CUPs
- Of the MS that notify the EMA of CUP, few have made use of the option to request a CHMP Opinion on conditions for use, the conditions for distribution and the patients targeted for CU

CHMP Scientific Opinions on Compassionate Use to Date

Product	Country	Year
ledipasvir, sofosbuvir	Ireland	2014
daclatasvir	Sweden	2013
Sofosbuvir	Sweden	2013
Zanamivir	Sweden	2010
Oseltamivir phosphate	Finland	2010

- Not transparent in which countries the CHMP Opinion led to the availability of the product under CU

Distribution of newly national initiated CU notifications



The graph only shows new notifications of investigational medicinal products received by EMA per year and does not include any renewals or end of CUPs notifications.

MS notifications received for investigational medicinal products for the same condition by more than one MS

Products	Disease/Indication
Regorafenib	Gastrointestinal Stromal Tumors
Enzalutumide	Castration resistant prostate cancer
Bedaquiline	(MTB) Pulmonary Infection
Teriflunomide	Multiple Sclerosis
Dolutegravir	HIV-1
Zanamivir	Life-threatening influenza infection
Abirateron-acetate	Castration resistant prostate cancer
Ibrutinib*	CLL and/or SLL and Relapsed or Refractory Mantle Cell Lymphoma
Daclatasvir	Hepatitis C
Ramiricumab	Gastric or gastroesophageal adenocarcinoma
Ceritinib	ALK-positive NSCLC
Alectinib	ALK+ NSCLC
Cobimetinib	Unresectable locally advanced stage IIIC or IV metastatic melanoma

Yellow highlights CHMP compassionate use opinions;
*also includes named patient programme

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Thank you for your attention

Further information

[Insert relevant information sources or contact details as applicable.]

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