A five-years surveillance of drug compassionate use in a University Hospital in Rome

CPC094

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Background

National compassionate use program are governed by individual Member States (MS) legislation. The Italian Ministerial Decree 8 May 2003, as amended in 2008, on the therapeutic use of an investigational medicinal product, represents the legislative frame defining a Compassionate Use Program (CUP).
Accordingly, an investigational drug can be requested to the manufacturing Company for use outside clinical trials, for a single patient or a cohort, if alternative treatments are not available. Safety and efficacy data from completed or ongoing phase III trials - in life threatening condition, data from completed phase II - must be available and fully comparable with the proposed patient(s) profile and a positive opinion be adopted by the local Ethics Committee (EC). Expanded access can also be part of CUPs.

Purpose

The aim of this surveillance was to achieve a clear picture of the CUPs at Hospital level. The outcome as subsequent Marketing Authorization (MA) of the involved products was also considered.

Materials and Methods

We analyzed data on the CUPs reported in our local Clinical Trial database, managed by a Pharmacist in charge of the EC Scientific Secretariat, in the timeframe between July 2006 and July 2011.
The following main parameters were considered: active substance, ATC, number of patients, therapeutic indication, subsequent MA.

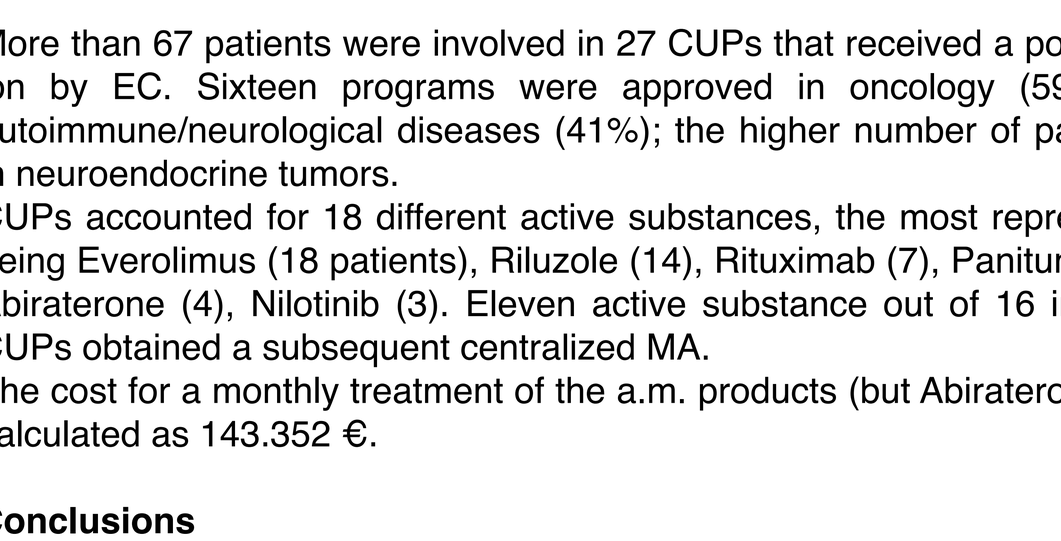
Results

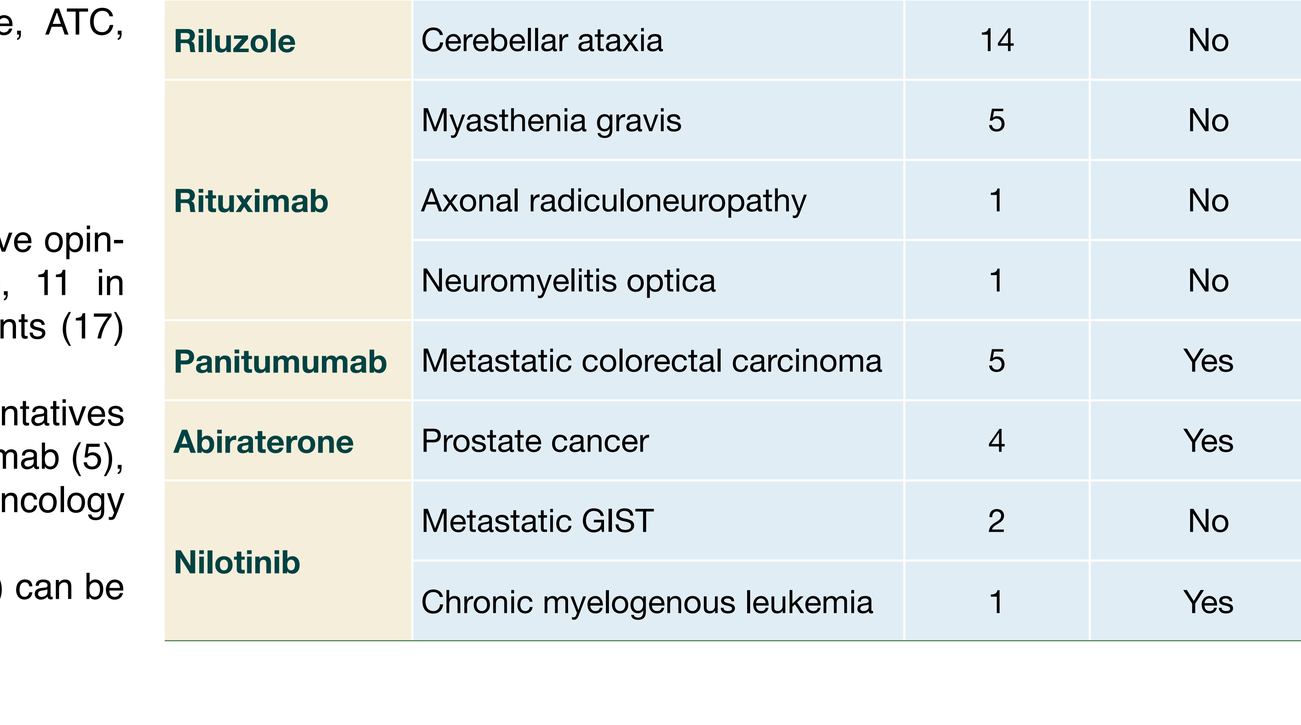
More than 67 patients were involved in 27 CUPs that received a positive opinion by EC. Sixteen programs were approved in oncology (59%), 11 in autoimmune/neurological diseases (41%); the higher number of patients (17) in neuroendocrine tumors.
CUPs accounted for 18 different active substances, the most representatives being Everolimus (18 patients), Riluzole (14), Rituximab (7), Panitumumab (5), Abiraterone (4), Nilotinib (3). Eleven active substance out of 16 in oncology CUPs obtained a subsequent centralized MA.
The cost for a monthly treatment of the a.m. products (but Abiraterone) can be calculated as 143.352 €.

Active substance	Therapeutic indication	Patients' number	Marketing Authorisation outcome
Everolimus	Gastro-intestinal neuroendocrine tumor	9	No
	Pancreatic neuroendocrine tumor (pNET)	6	Yes
	Neuroendocrine tumor lung primitivity	2	No
	Metastatic Renal Cell Carcinoma	1	Yes
Riluzole	Cerebellar ataxia	14	No
Rituximab	Myasthenia gravis	5	No
	Axonal radiculoneuropathy	1	No
	Neuromyelitis optica	1	No
Panitumumab	Metastatic colorectal carcinoma	5	Yes
Abiraterone	Prostate cancer	4	Yes
Nilotinib	Metastatic GIST	2	No
	Chronic myelogenous leukemia	1	Yes

Conclusions

CUPs may be offered as therapeutic options to patients in settings characterized by unmet medical needs. They can also represent a cost-saving opportunity at Hospital level.





Active substances and number of patients involved in CUPs