Reviewer’s report

Title: Patient organization involvement and the challenge of securing access to treatments for rare diseases: report of a policy engagement workshop

Version: 0 Date: 15 Mar 2017

Reviewer: J Jeppensen

Reviewer’s report:

Dear Koichi Mikami and Steve Sturdy

Thank you for the opportunity to read an interesting report.

First I have a few general and 'spontaneous' comments. Last I suggest some minor clarifications.

From my point of view, we need to secure a more independent evaluation of the efficacy/effect of new drugs. It is as if you take for granted that once a drug has been declared 'efficient', this is true. But a fundamental problem is that for most treatments there are a number - and often quite serious - side effects. Effective treatments almost always also effect negatively; and consequently difficult dilemmas are inherent in the development of treatment. The 'effect' can be contested with sound arguments, from both sides, proponents and opponents.

Soemhow we need to incorporate a critical medical science scientist in the process of development and final marketing of drugs (even the EMA is not critical enough, they can't be, as much personnel is recruited from the drug industry).


[There are no page numbers on my version of your manuscript, so the numbers I indicate below may vary from your version]

Line 16, p. 3: "Consequently, patients with seriously debilitating or life-threatening conditions may go untreated even though effective treatments are available."

This is very normal. There are many examples of patients that go untreated, first of all in poor countries/populations, but also in the Western world many poor, simply because the treatment is unaffordable (for many different reasons)

Main Text Method, Line 31, page 3: "… address the problem in a sustainable manner, …"

I lack a definition of what you mean by 'sustainable'.
Line 10, page 4: "... entrenched positions and animosities are avoided."

What could be examples of such positions and animosities?

Line 39, page 4: "Chatham House rules".

What are these rules? I think they are unknown to many non-British people. I myself never heard of them.

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I was reimbursed travel expenses to a joint meeting between the charity organisation Parents Project Muscular Dystrophy and the drug company PTC Therapeutics. I represented the Danish Neuromuscular Patient Association and the national Danish Rehabilitation Center for Neuromuscular Diseases
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