From: Jennifer Kelly []

To: SANTE PHARMACEUTICALS B5

Public Consultation on the Paediatric Medicines Regulation

These responses come from the Grace Kelly Ladybird Trust, registered charity 1167783 in response to the questions on the public paediatric medications regulation.
The Grace Kelly Ladybird Trust was set up in memory of 4 year old Grace who was diagnosed with a renal malignant rhabdoid tumour and died just 17 days after first showing symptoms. Her tumour type has an abysmal survival rate of less than 18% of children surviving 18 months. The main reason for this the rarity of the tumour making it unattractive to the pharmaceuticals industry. As a result, there has been very little research into this tumour type. Adequate incentives and less waivers could have made the future very different for Grace.

Our answers are below:

They are grouped according to the question that they answer.

Consultation 1:
We believe we need more evidence based medicine paediatric drugs available , especially for childhood cancer.
Current legislation has meant that actually there has only been two new medications developed and licensed for children with cancer as a result of old legislation. We need to act now.

Consultation item 2:

There has not been a significant increase in paediatric medicines for cancer over the past years. We believe this is mainly as a result of the loophole waivers that the regulation offers when drugs are developed for adult illnesses that do not exist in children, but the in essence the treatments may be useful for a different condition. We need to look at the mechanism of action of the drug.

According to SIOPE 2 , between 2012 – 14 there have been:

- 214 class waivers discussed
- 72% for an oncology drug, from which 95% granted waivers (i.e. 147 drugs)
- 63% of drugs waived were relevant to paediatric malignancies

As a result, many potentially active oncology drugs have not been investigated.

Consultation 5:
We fully understand that cost is a huge factor, especially for small pharmaceuticals companies, and as such suggest proposed changes to the reward system that would create greater incentives forthe industry and produce better outcomes for children with cancer.

The current system is not attractive enough to encourage drug development in many areas of paediatric medicine. We suggest bigger incentives are needed and more quickly.

The team at the Grace Kelly Ladybird Trust agree with
Professor Andy Pearson and colleagues from ACCELERATE, who points out that

"whilst the Regulation has brought positive change and advances, the waiver mechanism

means that with over 60% of 89 potentially valuable anticancer drugs granted a waiver, there

are still few paediatric trials and only between 9% and 15% of all oncology agents have

ongoing paediatric studies."

The Grace Kelly Ladybird Trust believes that children's lives should not depend on the goodwill of members of the pharmaceutical industry, a 'privilege' that can very easily be withdrawn.

Consultation 16:

We support view of SIOPE that paediatric oncologists providing their expertise should be paid for

their work.

Consultation 17:
We would like the access to trials for adolescents to be reviewed as the number of trials available to adolescents currently is actually less than children.

- Currently adolescents are grouped with children, so excluded from adult trials
- Adolescents shown to have similar tolerance to toxicity as adults

Inclusion in trials should be based on medical need rather than arbitrary age limits.

We at the Grace Kelly Ladybird trust support the recommendations in the Position Statement by SIOPE, Unite2cure and Cancer 13

Research UK:

1. Ensure that the *obligation* to undertake a Paediatric Investigation Plan is based on how a drug works and its capacity to address an unmet medical need in children - rather than the type of disease in adults for which it is first introduced.

2. Set up a mechanism to choose the best potential drugs and prioritise, among drugs developed by different companies, in relation to the real needs of children affected by rare cancers.

3. Reduce delays in paediatric medicines reaching children by enabling Paediatric Investigation

Plans to start not later than the start of pivotal trials in adults, if paediatric biological,

preclinical and preliminary clinical data are available to better evaluate the potential

therapeutic benefit in the paediatric population.

4. Add provisions for more effective and flexible rewards for companies undertaking early and

timely Paediatric Investigation Plans and those researching therapies specifically for cancers

which only occur in children

To this, we would add a further item however, the

Introduction of flexible ages of entry to adult trials based on considerations of biology and safety.

Many thanks, yours sincerely

Dr Jennifer Kelly

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