ACCELERATE

INNOVATION FOR CHILDREN AND ADOLESCENTS WITH CANCER

Everything you always wanted to know about Drug Development for Children with Cancer (but were afraid to ask): Poll slides

ANDREW McDONOUGH B+ FOUNDATION®

Learning objectives

2. How under-served are children?

Poll 1

Carboplatin is one of the most used drugs in standard treatment in pediatric oncology.

Which of the following is true?

A. Its pediatric use was approved in 1998 by the FDA and in 1999 by the EMA
B. The pediatric dose recommendations have been well established and sanctioned by the competent authorities
C. Carboplatin has no approved pediatric indication and therefore is prescribed solely off-label
D. There are few data available regarding safety, effectiveness and dose recommendations in children

Multiple answer possible

Answer

Carboplatin is one of the most used drugs in standard treatment in pediatric oncology. Which of the following is true?

A. Its pediatric use was approved in 1998 by the FDA and in 1999 by the EMA **WRONG**

B. The pediatric dose recommendations have been well established and sanctioned by the competent authorities **WRONG**

C. Carboplatin has no approved pediatric indication and therefore is prescribed solely off-label **TRUE**

D. There are few data available regarding safety, effectiveness and dose recommendations in children **WRONG**

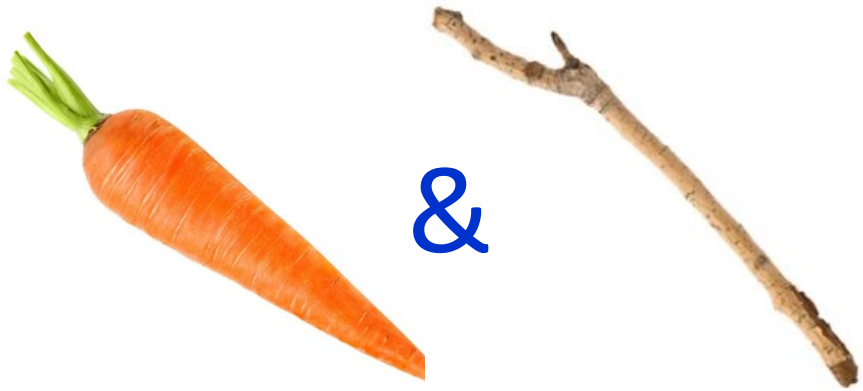
Poll 2

Should companies selling carbopatin be obliged to submit data for marketing authorization for the treatment of pediatric malignancies?

A. YES
B. NO
C. I do not know

Learning objectives

3 Regulations tried to help: success?



Poll 3

Under the Best Pharmaceuticals for Children Act:

A. A Written Request is issued by FDA
B. A Written Request contains study(ies) for only one indication
C. A company cannot ask for a Written Request
D. A PREA requirement and a Written Request can be issued for the same medicinal product

Answer

Under the Best Pharmaceuticals for Children Act:

- A Written Request is issued by FDA **TRUE**
- A Written Request contains study (ies) for only one indication **WRONG**
- A company cannot ask for a Written Request **WRONG (PPSR)**
- A PREA requirement and a Written Request can be issued for the same medicinal products - **TRUE**

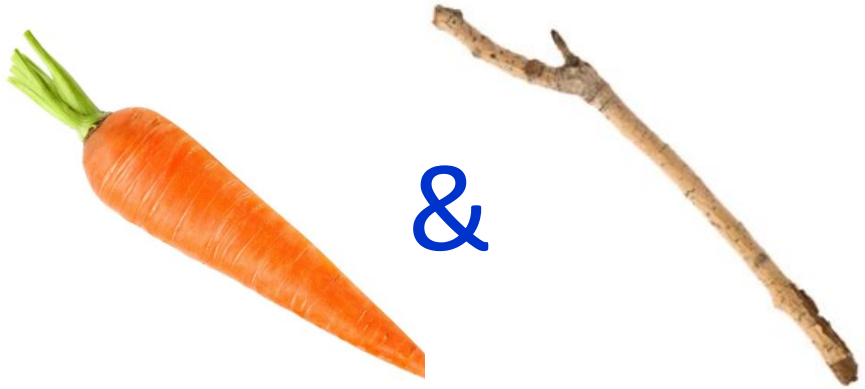
Poll 4

Is the Reward attractive for Pharma?

A. Yes
B. No
C. It depends
D. I do not know

Reward

3 Success?



Poll 5

How many new medicines/new indications have been authorized in the US and EU between 2008 and 2019?

For childhood cancers:

A. Less than 20
B. 20 to 40
C. More than 40

For cancers in adults:

D. Less than 50
E. 50 to 150
F. More than 150

Answer

- How many new medicines/new indications have been authorized in the US and EU between 2008 and 2019?
- **18 anticancer medicines** with a pediatric indication
  - **15** in the **US**
  - **12** in the **EU**
  - Including **10** in both the **US** and **EU**
- Versus **MORE THAN 150** in adults

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*new molecular entities

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Poll 6

Will the new regulatory environment accelerate the development of safe and effective anticancer drugs for the treatment of childhood and adolescenthood malignancies?

A. Sure
B. Not Sure
C. No
D. I need more information

A Clinical case

CLINICAL CASE

1) Do you think Grunitinib should be investigated for the treatment of minionblastoma?
   A. No, minionblastoma already has a well-established frontline treatment with high survival rates
   B. No, the minimum cost-effectiveness standards are not met
   C. No, Grunitinib does not need to be investigated for this indication – it should be used in children with minionblastoma off-label right away
   D. Yes, this is a potential indication that should be explored, beginning with a first-in-child clinical trial

CLINICAL CASE answer

1) Do you think Grunitinib should be investigated for the treatment of minionblastoma?
   A. No, minionblastoma already has a well-established frontline treatment with high survival rates **WRONG**
   B. No, the minimum cost-effectiveness standards are not met **WRONG**
   C. No, Grunitinib does not need to be investigated for this indication – it should be used in children with minionblastoma off-label right away **WRONG**
   D. Yes, this is a potential indication that should be explored, beginning with a first-in-child clinical trial **TRUE**

CLINICAL CASE

2) If you were the company owning the compound, what regulatory process would be mandatory for you to submit in the USA under the RACE Act?

A. iPSP
B. PPSR
C. Waiver
D. PIP
E. None of them are mandatory

CLINICAL CASE answer

2) If you were the company owning the compound, what regulatory process would be mandatory for you to submit in the USA under the RACE Act?

A. iPSP **TRUE**

B. PPSR **WRONG**

C. Waiver **WRONG**

D. PIP **WRONG**

E. None of them are mandatory **WRONG**

CLINICAL CASE

3) What will need to be granted at the time of filing in adults in the EU?

A. iPSP
B. PPSR
C. Waiver
D. PIP
E. None of them are mandatory

CLINICAL CASE answer

3) What will need to be granted at the time of filing in adults in the EU?

A. iPSP **WRONG**
B. PPSR **WRONG**
C. Waiver **WRONG**
D. PIP **TRUE**
E. None of them are mandatory **WRONG**