

Nursing to regulator, rowing to digital health

Elin H Davies speaks to Stella Bennett, Commissioning Editor: Elin qualified as a children's nurse at Great Ormond St Children's Hospital, pursuing a BSc, MSc and receiving a PhD from University College London. Elin has over a decade of clinical and academic experience of research and drug development, working at an international level. She also worked at the EMA for 6 years as part of the team responsible for implementing the Paediatric Regulation in Europe. In January 2015, Elin launched a social enterprise using wearable technology and mobile phone apps to monitor disease progression. She also has a personal interest in empowering children to become involved in the health/research agenda. Her personal passion is for extreme challenges and adventures.

Elin Haf Davies, Director: *Enabling Research*

Q Tell us a little about your personal journey from pediatric nurse to campaigner & clinical trial consultant?

I guess it has been an interesting journey, and I probably would have never guessed that my career would have taken the turns it did! I always knew I wanted to be a children's nurse. I went to volunteer for Save the Children in Lesotho, Africa before I started my nurse training, and working with children and child health was all that I have ever wanted to do, really. I cannot say that I specifically, carefully planned out my career choices but I certainly took every opportunity that I could, and found that research, sort of, stimulates me in trying to get new ways to improve the lives of the children I have cared for over the years.

Q How would you describe the work that you do on clinical trial design?

Because of the clinical experience I had, of trying to implement clinical trials that were just not practical for children to do, in that, they were really invasive and emotionally exhausting for the children and family alike, my number

one goal is 'can we design clinical trials that are friendlier for children, that are easier to implement, and have value in terms of the day-to-day life that they are having to live and not just the snapshot hospital visits – but also making scientific advancement at the same time.'

Q What would you say are the challenges specifically faced in designing pediatric clinical trials, & what solutions do you propose?

I guess the biggest challenge is that the usual powered sample size calculations don't really apply here, and what adds to the complexity and makes it even harder is that these diseases are really heterogeneous in presentation and progression, so there is a wide variation in the symptoms and the severity that they present with. And then, obviously, you find that you need to monitor them for a very long time, and clinical trials are not really designed to run for more than 12–24 months. So, you do not really have the longevity that is needed to monitor them.

Q From your webpage, it is clear that you are passionate about raising awareness of rare or orphan diseases. What do you think is necessary to



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conduct clinical trials when the affected population is so small?

It is complete patient engagement and patient empowerment. Before you even start, it is understanding what the patient's needs are, and what is important to them in their day-to-day life. I think we are finally now realizing that we should protect children through research, not protect children from research. Very importantly, if a clinical trial is going to work, if a drug is going to give us value for money, it needs to have the patient voice at the heart of the design [1].

Q You discuss patient empowerment and patient-centricity in clinical trials: do you think this approach is the future of clinical trial design?

Absolutely! One of the key things that I am quite passionate about is trying to change the communication culture within the area. At the moment, it is a very one-way dialogue between all the different stakeholders, so the industry will speak to regulators, patients will speak to the doctors, doctors will speak to the industry, but actually everybody joined up and talking together hardly ever happens. Then, you get a lot of Chinese whispers and misrepresentation of what people have said, so I really believe that we need to change the communication culture and get everybody talking together at the same time and much earlier.

Q In a recent talk, you discussed the use of Medicine Adaptive Pathways for Patients. Could you briefly describe what this means, & why it is a necessary move from adaptive licenses?

Adaptive licensing is primarily about convincing the regulators that there are different needs to the trial designs other than the ones we go for as standard, and that a different approach from a regulatory point of view is needed to get drugs to patients with high needs quicker. With Medicine Adaptive Pathways for Patients (MAPPs), it encompasses far more, so it encompasses what the patient's views are about taking uncertainty and accepting the risk, allowing the

patient to be more empowered in the decision-making process. But crucially, it also brings in the payer to discuss what the value for money is, and how these drugs are going to be reimbursed. So, I would say that it is moving away from a just an industry-regulator dialogue into a much wider approach where we get all the stakeholders together making joint decisions.

Q What do you believe the future holds for clinical trial design, particularly for chronic or rare conditions?

I generally think there are two really big things that will change the face of drug development in orphan diseases. One of them is MAPPs; I think that MAPPs will allow quicker access to the patients who need it and an easier pathway for innovation to be utilized. We all know that the cost of developing drugs is getting out of control in rare diseases, and we also know that the healthcare system cannot afford to pay for the drugs that we already have in place, so something needs to change. Reducing the drug development price might also, hopefully, mean that we could get it to patients cheaper, but at a benefit risk that makes it worthwhile to everybody involved. I think one of the innovations that will harness that is mobile health and wearable technology. I am part of a social enterprise called aparito [2] and I think that the use of continuous real-world data, of how the patient is doing in their day-to-day life will be far more informative in telling us whether the drug works, whether it has efficacy and effectiveness, and whether it is of good value.

Financial & competing interests disclosure

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No writing assistance was utilized in the production of this manuscript.

References

1 Elin Haf Davies: empowering children, enabling research. www.elinhafdavies.co.uk

2 Aparito. www.aparito.com