



A practical solution to real issues patients face in DMD trials

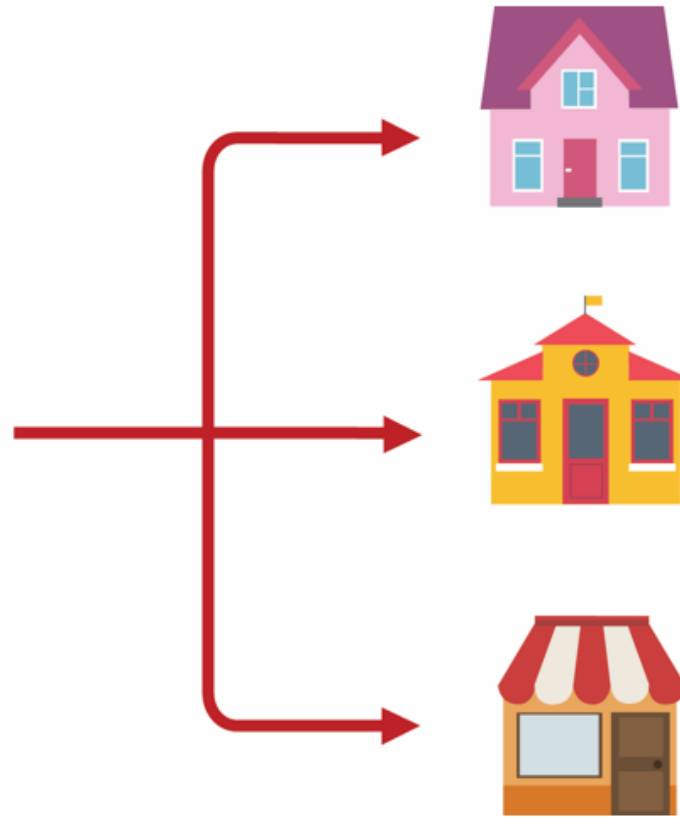
Helen Springford, Vice President Strategic Development
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What Illingworth offers?

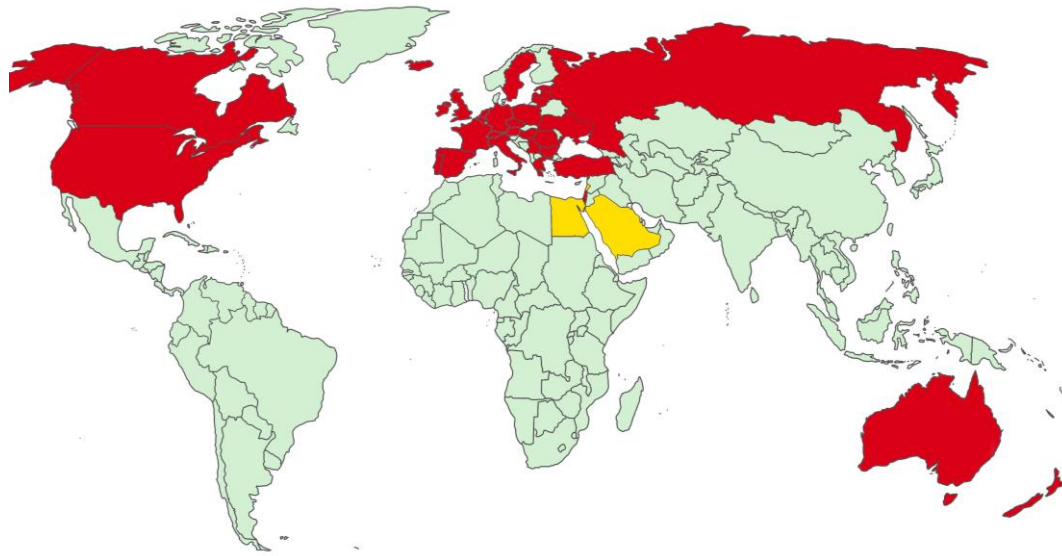
On-Site Site Support



Off-Site Patient Visits



Illingworth's global footprint



Red - Illingworth Research Nurses

Yellow - in development

Region	Countries covered
North America	USA, Canada
Europe	Austria, Belgium, Bulgaria, Czech Republic, Denmark, Estonia, Finland, France, Germany, Hungary, Iceland, Ireland, Israel, Italy, Latvia, Lithuania, Netherlands, Norway, Poland, Romania, Russia, Serbia, Spain, Sweden, Switzerland, Turkey, Ukraine, United Kingdom
Australasia	Australia, New Zealand
Middle East	Israel Egypt and Jordan - in development

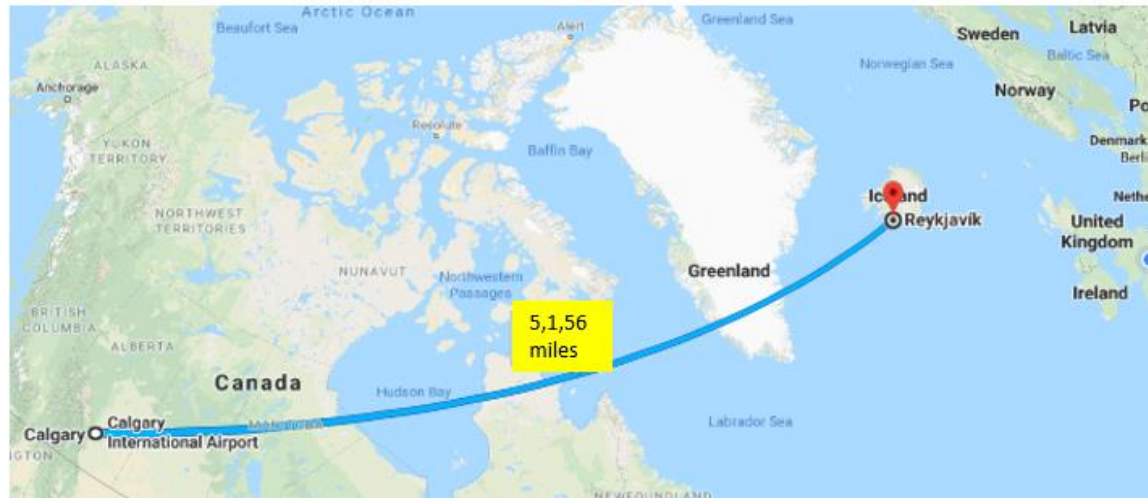
Case Study- Setting the Scene

- The family live in Reykjavik, Iceland but there are no sites participating in the clinical trial there.
- The family approach a site in Calgary, Canada. The site team screened the brothers, both diagnosed with DMD for the clinical trial.
- Both children were enrolled into the study.
- The children have to attend on-site clinic visits in Canada at weeks -4, -2, 0 then every week for the first 8 weeks, then once a month from week 8.
- Visits at weeks 2, 3 and 6 only required a blood sample.
- The protocol also required unscheduled visits for repeat safety bloods.
- Consider the impact on the children and the rest of the family.....

Considerations



Patient Travel... the reality



No direct flights available

A minimum of 11 hours flight, not including travelling to the airport, waiting for the flight and transport upon arrival. A specialist travel company was contracted to support the family.

The family require at least 3 days per week in Canada to allow weekly visits at the site.

The Solution

- Illingworth identified an experienced research nurse within walking distance to the boys' home in Iceland.
- The nurse was fully trained on the protocol, signed the Canadian site delegation log remotely so that she became an extension of the study site.
- The nurse went to the home to take blood and process it in the home at visits 5, 6 and 8 which meant that the family did not need to fly back to Canada for those visits.
- Our nurse was easily accessible to conduct unscheduled blood samples, which would have been impossible to arrange without a mobile research nurse.

Paediatric Trials



Child friendly materials including stickers



The impact...

We asked the Mum to provide some feedback and this is what she said;

Quote from the family

“There were a few areas where getting off site nursing for the blood test was very beneficial for us.

First it was great for the boys, to get some much needed break from travel, instead of missing school, missing out on seeing friends due to being away. Having the blood test done back home provided them with a much more normal week instead of having to travel.

For us, as a family, it was great to have everyone home and not disturb family life more due to one parent and a child being away for days at a time. This also greatly benefitted our two younger daughters, making life as normal as possible. They didn't notice anything different about the weeks where we got home care nursing.

And for us, the parents, no travel meant a normal work week and not needing any days off.

Overall we were very happy that it was a possibility and it was a very positive experience for all of us.”

In summary...

Including off-site visits to study protocols can have a very positive impact on all concerned;

- Sponsors have a more attractive protocol which in turn attracts more potential subjects. There is often choice as to which trial a subject can enter. Making it as patient focused as possible will increase the chances that subjects will choose your trial over the next one.
- Allowing visits at home for those who live miles away from site increases the potential pool of study subjects by expanding the geographical reach.
- Sites have more time to concentrate on enrolling more subjects as the more basic visits not requiring specialist equipment and physician involvement is performed off site.
- Families find it much less stressful.

Any Questions?



Thank you
for your time.