President's Message

Writing for rare diseases

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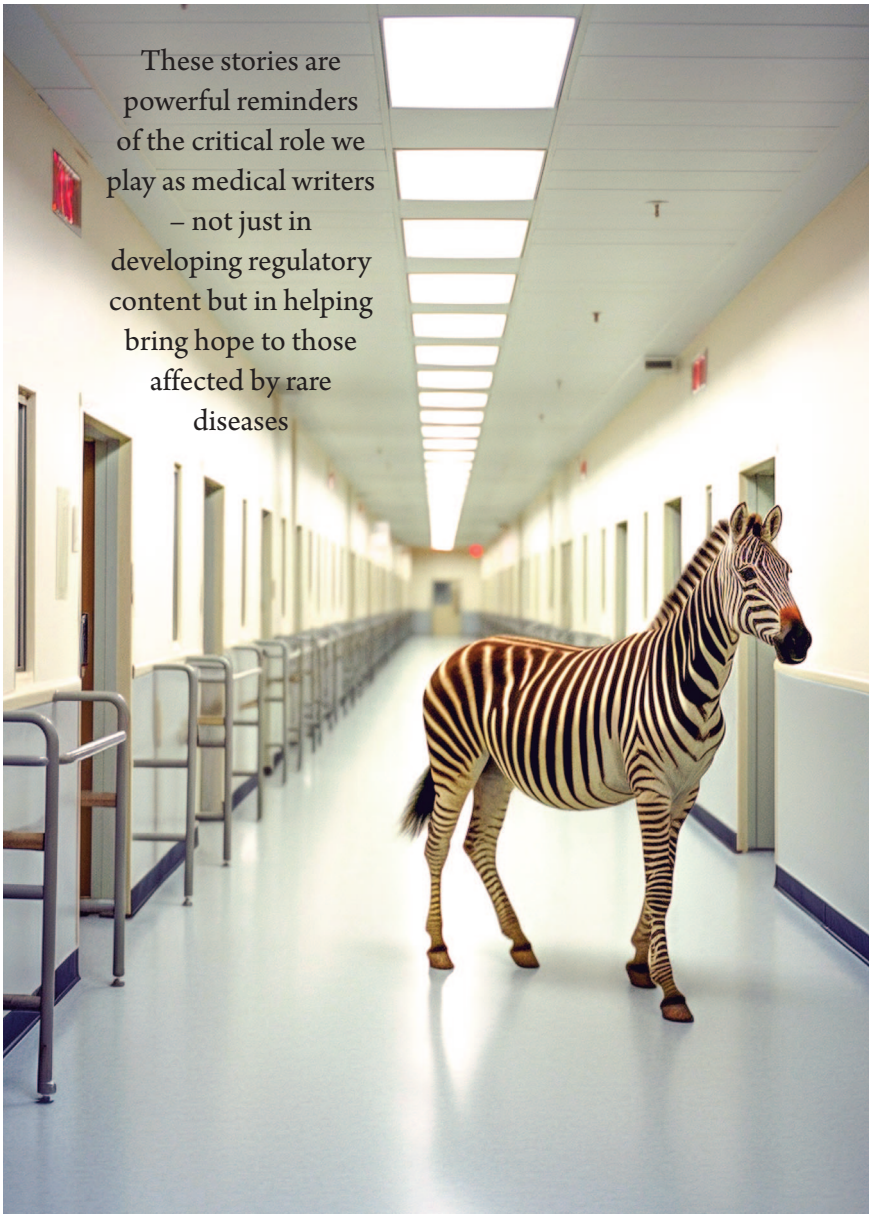
"When you hear hoofbeats, think horses – not zebras."

Dear EMWA Colleagues,

This familiar adage is often taught to medical students to encourage simple, common-sense diagnoses. The idea is that the most straightforward explanation is usually correct. However, while it's a useful rule of thumb, it also risks overlooking the reality that "zebras" – rare diseases – do indeed exist.

In our increasingly globalised and interconnected world, it's easy to assume that modern medicine has all the answers. Imagine, for example, the awe of ancient Romans when Julius Caesar introduced the giraffe to Europe in 46 BC, with many believing it to be a cross between a camel and a leopard. Today's rare diseases – though certainly better understood than the giraffe was to the Romans – still present significant diagnostic and treatment challenges for medical professionals trained to expect the "horses" of common conditions.



I've seen this play out personally. For many years, my father-in-law was prescribed various treatments for persistent eczema, without relief. Only after an off-hand remark from a friend prompted him to get tested for coeliac disease did the pieces fall into place. His diagnosis led to the lifestyle changes that finally improved his condition.

On a professional level, I've also had the privilege of working on regulatory documents for novel products for the treatment of rare diseases. Reading patient testimonials about lives transformed by access to new therapies provided a sense of purpose and deep motivation. These stories are powerful reminders of the critical role we play as medical writers – not just in developing regulatory content but in helping bring hope to those affected by rare diseases.

In this issue of *Medical Writing*, we honour the zebras. Kelley Hill shares a career's worth of insights on regulatory writing for rare diseases. Sarah Milner interviews Richard Farquhar, who offers a candid look at living with a rare condition. Cheryl Roberts tackles diversity in trials for rare diseases, while Philip Burridge and Julie Eastgate discuss how master protocols can expedite such clinical studies. Christian J. Hendriksz discusses the pivotal role medical writers can play in providing hope to rare disease patients. Together, these stories and insights remind us of the profound impact we can have in helping turn the extraordinary into the possible.

Enjoy this issue and thank you for joining us in highlighting the vital work being done for those who live with rare diseases.