

An ultrarare



Alexion's Soliris, a drug which has won awards for medical innovation, has earned its commercial stripes, too. At press time, the biologic was on track to cross a billion dollars in worldwide sales. That's not so rare among therapies that, like Soliris, treat extremely obscure ailments and cost \$200,000 or more per patient per year, but it's impressive considering the patient pool is just a few thousand.

The drug, Alexion's first and only commercialized product, has helped push the company into the biotech big leagues with a market cap just shy of \$20 billion, the sixth-largest behind names like Gilead and Amgen. Analysts expect Soliris to become a multi-billion dollar franchise.

How it got to this point is a model for building a profitable ultra-orphan brand. "We're focused on developing and delivering therapies that transform the lives

L-R: Jeroen van Beek, VP, global nephrology franchise; David Hallal, EVP, chief commercial officer; Margaret Olinger, VP, global hematology franchise, Alexion

complement-mediated
SES sudden and
e **ORGAN FAILURE**
nature **DEATH**

ypical Hemolytic Uremic Syndrome (aHUS).
ntrolled complement activation leads to systemic
mbiotic microangiopathy (TMA), causing progressive
age to vital organs and premature death¹⁻³

ALEXION

success story



Alexion education efforts about Soliris (below) include non-branded disease sites for aHUS (above) and for PNH. Below left, a disease awareness program

of patients with ultrarare and severe, life-threatening diseases—patients that without our therapies have no other hope,” says Alexion’s David Hallal, EVP, chief commercial officer.

When *MM&M* caught up with Hallal in early December, his team was in the midst of launching Soliris for the second of its two indications, atypical hemolytic uremic syndrome (aHUS), approved in late 2011. Approval for the first, paroxysmal nocturnal hemoglobinuria (PNH), was secured in 2007. “In these settings, Soliris has a 100% response rate,” he says.

The US aHUS launch is progressing well, according to analysts, with European reimbursement for aHUS expected in mid-2013. Several other indications are set to follow.

“Our approach to aHUS was similar to that of PNH,” notes Alexion’s Jeroen van Beek, PhD, VP, global nephrology franchise, “in that the focus is on disease education and establishing the importance and pathway to rapidly

diagnose patients with aHUS so that they can receive the life-transforming benefits of Soliris.”

In addition to a highly educated sales force calling on hematologists, nephrologists and renal transplanters, Alexion supports promotional programs where KOLs help teach physicians in the field. It also utilizes medical conferences, where its trade show booth focuses on early diagnosis and intervention.

While the firm maintains a disease, brand and corporate web presence, its most important patient-facing effort, says Hallal, is its One-Source education

AGENCY ROSTER

PROFESSIONAL (HEMATOLOGY)

McCann Regan Campbell Ward

PROFESSIONAL (NEPHROLOGY)

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DIGITAL

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SmithSolve

and reimbursement program, in which Alexion’s staff of registered-nurse case managers works one-on-one with the patient, family and HCP.

“It’s really important to advocate for that individual patient and not necessarily attack all payers with a broader central message,” he says. “Among payers, their experience is usually with a few [PNH or aHUS] patients anyway.”

Two-thirds of its business is ex-US, and the firm has secured public funding for Soliris in such jurisdictions as Australia and Canada—two countries well known for being perhaps even more restrictive than the EU. That’s not a slam dunk given that the drug costs about \$440,000 per patient per year (it’s sometimes given away, in cases of hardship).

“Because the benefits of Soliris are transformative and not incremental, governments around the world...are recognizing the benefit,” says Hallal.

For instance, he cited research suggesting that PNH patients followed for up to eight years on treatment can expect the same life span as those of a normal population that are age- and gender-matched. If left untreated, PNH has a 35% mortality rate at five years. Says Hallal: “This provides a very strong underpinning for our access strategy.”

Disease awareness and diagnostic programs are set to increase, adds Margaret Olinger, VP, global hematology franchise, in order to “positively influence the entire cycle of care.”

PNH and aHUS are forecast to achieve global sales peaks of about \$2 billion each, and with compelling evidence of Soliris efficacy in other indications, it could reach up to \$8 billion in peak sales, according to Barclays Research. Go to mmm-online.com for more creative. — Marc Iskowitz



PHOTO LEFT: BILL BERNSTEIN