

Rare Diseases // Overview of Capabilities

One of the challenges to enrolling clinical research studies of rare diseases can be finding eligible patients. The BBK solution is to work with study sites to provide the consultation, services, and technology they require while developing site-specific recruitment plans with specialized outreach and data-mining guidance for every step of the recruitment process.



// von Willebrand Disease (VWD)

Identifying and consenting 18 patients with severe VWD can be like finding a needle in a haystack. BBK often supports these types of recruitment campaigns – where there’s an extremely limited patient pool to draw from – by motivating and re-engaging participants who have taken part in previous research studies of the same condition.

MANAGING
PRIMARY
IMMUNODEFICIENCY

// Primary Immunodeficiency (PID)

There are more than 70 different types of primary immunodeficiency – some relatively common, others quite rare. When a research study requires participants to represent many of these different variations, BBK often provides site-by-site recruitment planning after determining the exact support each site is in need of most.



// Bleeding Disorders

From rare bleeding disorders to common trauma, BBK has successfully enrolled a number of global studies related to coagulants. Our experience has shown that for these studies, it is most effective to provide investigators and their staffs with educational materials to engage and inform those patients who know little about their condition.



// Immune Thrombocytopenic Purpura (ITP)

ITP has a low incidence among patients, so studies of this condition always need strategically placed sites. That’s why sponsors often bring their ITP studies to BBK – to benefit from our sophisticated processes for identifying, filtering, and ranking sites. Using these processes, BBK has identified a vast number of superior-ranked sites.



investigating enzyme replacement therapy
for infants under six months with pompe disease

// Pompe Disease

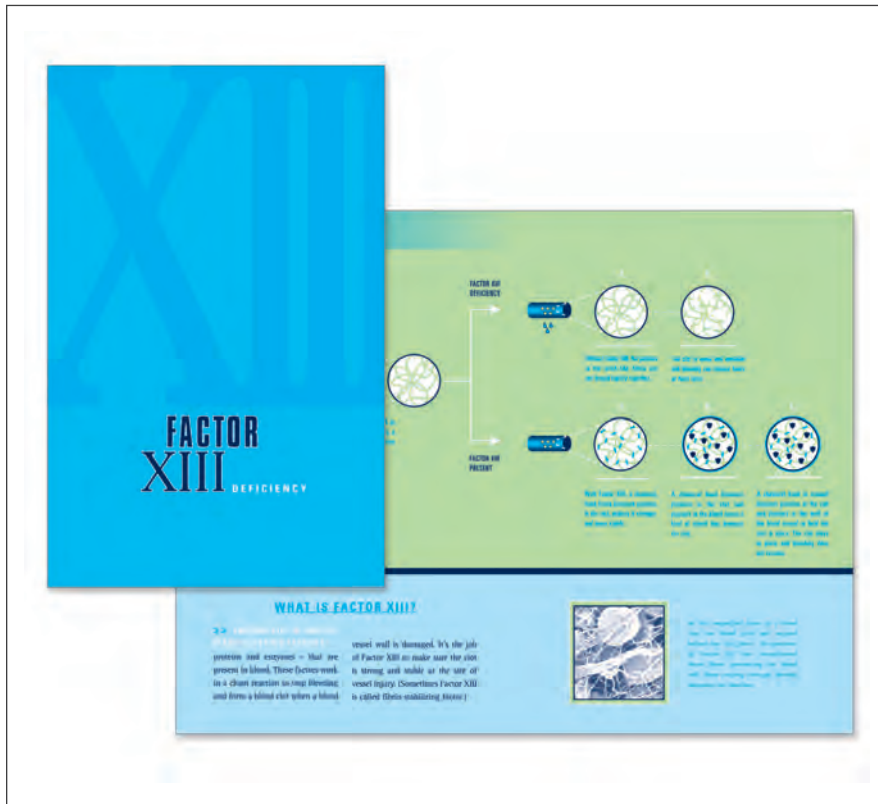
Clinical studies of rare conditions – may be considered unenrollable. Doctors don’t typically screen for a condition that affects less than 100 infants born each year, so finding eligible study participants is a challenge. By initiating a global physician-outreach campaign, BBK can educate a country’s medical community about both the disease and the study opportunity.



// Retinal Vein Occlusion (RVO)

BBK is keenly aware of the needs of patients who suffer from central and branch retinal vein occlusion. With RVO, the key is to create materials that are accessible to patients who are vision-impaired, as well as training site staff how to best communicate the study opportunity to an older population of patients.

Phase I Study // Factor XIII Deficiency



Silver Bullet // BBK's carefully written and well-designed study brochure provided sites with guidance on how to clearly introduce and explain the complicated condition, concerns with existing treatments, the potential risks and benefits of the investigational drug, and study participation – for both adult and pediatric participants.

OUR INVOLVEMENT	After First-Patient-In Date
COUNTRY	United States
AUDIENCES	Patients Parents Investigators Site Staff
KEY TO ENROLLMENT	Study Messaging

15 Total Sites

Most Difficult to Motivate

1

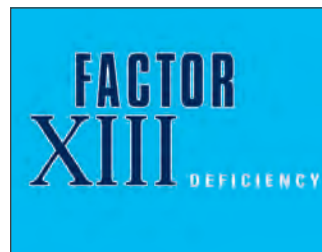
Number That Counts // One extremely effective tactic made the most of this sponsor's meager recruitment budget. What's more, the study brochure's savvy design helped minimize costs for the next protocols; the back-cover pocket housed an insert with protocol-specific participation information, which sites could easily print as needed.

Objective // Create recruitment material to help sites enroll 65 patients within 10 months for this Phase I study

Challenges // Complex condition and treatment made explaining study participation difficult; small recruitment budget

Results // Enrollment rate increased exponentially, helping to enable complete randomization; sponsor repurposed the study brochure for next phases of study

Expert Analysis



The sponsor for this study of a treatment of Factor XIII Deficiency – one of the rarest of bleeding disorders – had a meager recruitment budget and many patients to enroll. BBK's all-in-one solution was an optimally designed brochure that used simplified language, constructs, and illustrations

to introduce and explain the significant treatment option that study participation offered to patients. Anticipating the sponsor's need for materials for the next phases of study of the treatment, BBK designed the brochure so that it could be easily reprinted for subsequent protocols without a costly redesign. The well-crafted copy in this brochure was not only approved without edits, but also lauded for its clarity by the many academic IRBs monitoring this study.

Phase I/II Study // Pompe Disease



Enrolling Pompe Early // A referral-request program marshaled the help of department heads, a compelling brochure alerted physicians to the rare disease and the study opportunity, and useful reminder items kept the study top-of-mind: this simple, well-executed campaign enrolled this challenging study ahead of schedule.

OUR INVOLVEMENT

After First-Patient-In Date

COUNTRIES

France, Israel, Taiwan,
United Kingdom, United States

8 Total Sites

AUDIENCES

Parents
Heads of Pediatric Neurology Dept.
Neonatologists
Pediatricians

Most Critical to Motivate

KEYS TO ENROLLMENT

Department Heads' Endorsement
Global Referral Outreach

1

Number That Counts // Pompe is so rare that most neonatologists, neurologists, and pediatricians have never heard of it, and a pediatric neurology specialist may not see a single case in an entire career. But by asking physicians to rule it out first when a patient exhibits symptoms, BBK was able to enroll this landmark clinical trial – once thought by the sponsor to be “unenrollable” – ahead of schedule.

Objective // Enroll 50 patients with Pompe disease – a very rare condition occurring in approximately 70 newborns worldwide each year

Challenges // Hard-to-diagnose condition last to be considered by neurologists in diagnosing symptoms that are shared with more than 40 more common conditions; patients had to be enrolled soon after birth

Result // Enrollment achieved ahead of schedule

Expert Analysis

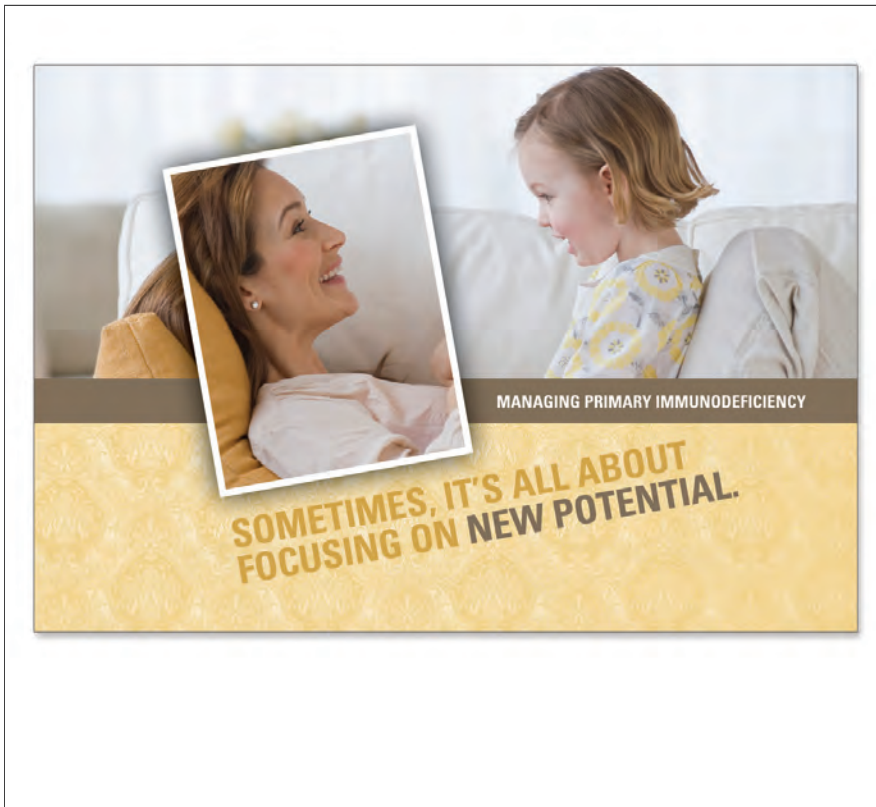
pompe STUDY

investigating enzyme replacement therapy for infants under six months with pompe disease

Study participation offered treatment for Pompe disease – an often fatal lysosomal storage disease typically found in newborns – where none existed. However, enrollment was exceptionally problematic because:

only about 70 cases throughout the world are diagnosed each year; the condition shares symptoms with other, more prevalent conditions and therefore is not first considered; and patients had to be enrolled within a short time following birth. BBK enrolled this global study ahead of schedule by initiating a global physician-outreach campaign that educated and marshaled the support of pediatric neurology department heads, their staff, and their referral networks in major medical centers throughout the world.

Phase III Study // Primary Immunodeficiency



Statistical Complications // This sponsor was evaluating a promising reformulation of a treatment for many different types of primary immunodeficiency. But to do so, the sponsor had launched a study statistically dependent on including adult patients, pediatric patients, patients with no treatment history, patients with specific treatment histories, patients with particular primary immunodeficiencies... BBK knew enrollment would be a challenge before the end of the list.

OUR INVOLVEMENT	
After First-Patient-In Date	
COUNTRIES	
United States	} 16 Total Sites
Canada	
AUDIENCES	
Site Staff	} Most in Need of Support
Adult Patients	
Parents/Guardians of Pediatric Patients	
KEY TO ENROLLMENT	
Site-by-Site Recruitment Planning	

70

Number That Counts // There are about 70 different kinds of primary immunodeficiency – and this study’s statistical method depended on patients representing many of these different variations of the condition. In the end, enrollment depended on BBK’s ability to orchestrate the study’s enrollment one site – and one patient – at a time.

Objective // Help study staff identify and recruit only 80 patients total – but using almost a dozen different subsets of eligibility criteria

Challenge // Study needed specific subgroups of patients who fell within the full eligibility criteria

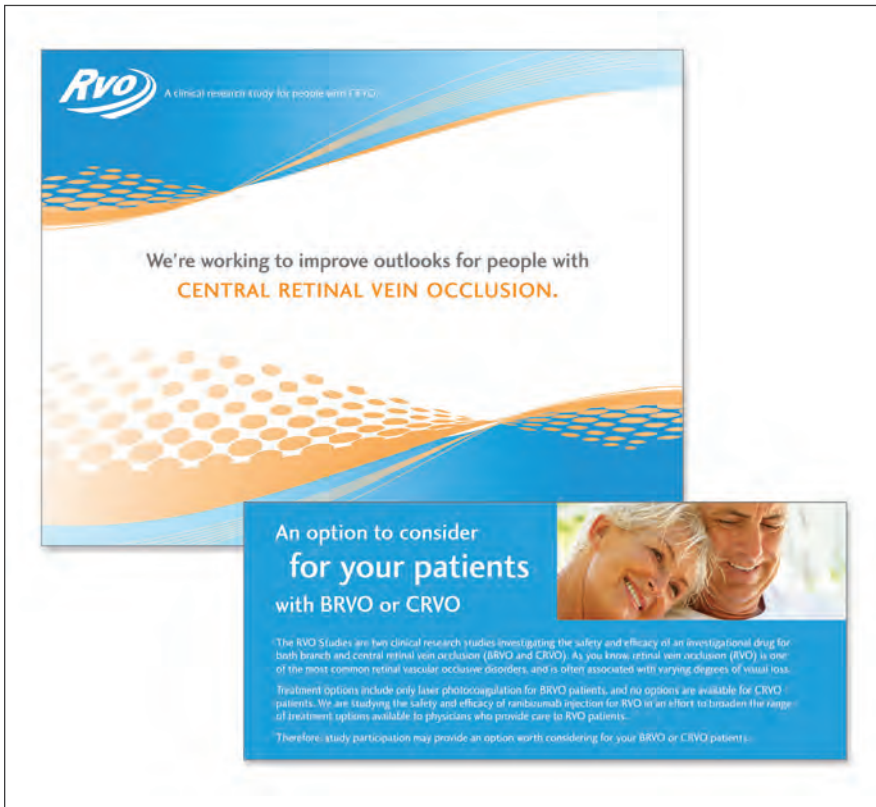
Result // Enrollment finished with months to spare after BBK’s recruitment planning gave the sites the guidance and direction they needed

Expert Analysis

MANAGING PRIMARY IMMUNODEFICIENCY

The sponsor hoped its streamlined investigational therapy could ease the time-intensive burden of managing primary immunodeficiency. But to cover as many different patient populations as possible, the sponsor’s study demanded specific subgroups of eligible patients. Once those requirements slowed enrollment to a crawl, BBK was called in. After in-depth analysis of each site’s recruitment situation, BBK triaged directly with staff to determine the exact support each site needed most. These analyses and interviews led to site-specific recruitment plans that provided specialized outreach and data-mining guidance for every step of the enrollment process. When the sites relaunched their recruitment efforts with BBK’s plans in hand, enrollment whisked to an early close.

Phase III Study // Retinal Vein Occlusion (RVO)



Building Referral Networks // To promote this sponsor's two retinal vein occlusion studies involving frequent eye injections, BBK increased study awareness by reinvigorating referral networks, creating site- and patient-facing materials, and providing consultation to sites to overcome study barriers – resulting in full enrollment weeks ahead of schedule.

OUR INVOLVEMENT	
After First-Patient-In Date	
COUNTRY	53 BBK-Supported Sites; 213 Total
United States	
AUDIENCES	
Patients	Most Critical to Motivate
Referring Physicians	
Site Staff	
Investigators	
KEY TO ENROLLMENT	
Physician Referral Program	

5

Number That Counts // With approximately five months remaining, BBK helped this sponsor fully enroll both studies – a combined five weeks ahead of schedule.

Objective // Enroll a total of 780 patients for two studies at sponsor-selected sites within five months

Challenges // Condensed enrollment time line; multiple ocular injections

Result // Study enrollment for both studies achieved ahead of schedule

Expert Analysis



The sponsor's initial recruitment vendor chose a direction that failed to create a compelling motivation for patients to participate, was unable to engage referring physicians, and overlooked the chance to position both

studies as part of a larger effort toward improving treatment for RVO. BBK responded by creating a unified study brand that leveraged these studies as unique opportunities to address a condition with few effective treatments. BBK also initiated a referral request campaign that provided a wealth of eligible study participants, and designed additional communication materials to address enrollment barriers and educate patients about RVO and its competing treatment options.

Phase I Study // von Willebrand Disease (VWD)



Meeting the Challenge // BBK has established an impressive track record helping study sites successfully enroll research studies of rare conditions – such as von Willebrand disease. The BBK solution is to create site-specific recruitment plans and then provide the consultation and resources sites need to effectively implement these action plans.

OUR INVOLVEMENT

Before First-Patient-In Date

REGIONS

Europe

North America

} 20 Total Sites

AUDIENCES

Site Staff

Patients

Physicians

} Most in Need of Support

KEY TO ENROLLMENT

Site-by-Site Recruitment Planning

18

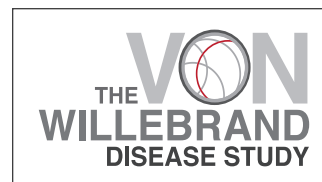
Number That Counts // Although this research study required only 18 participants, recruiting that number in just four months was proving to be a major challenge for sites. BBK was brought in to quickly assess the study landscape and capabilities of sites, and created site-specific recruitment plans – with specialized outreach and data-mining support and consultation – to help study staff achieve on-time enrollment.

Objective // Support the enrollment of 18 patients with severe VWD in four months

Challenges // Limited patient population; no therapeutic benefit; time commitment

Result // On-time enrollment achieved at all 20 study sites

Expert Analysis



BBK's extensive experience coaching site staff to identify and consent patients for research trials of rare diseases is one of the many reasons the sponsor of this Phase I dose escalation study of VWD procured our

services. Based on the findings of a comprehensive survey of each of the 20 sites and their capabilities, BBK provided study staff with recruitment consultation, services, and technology to help them achieve on-time enrollment. On the creative side, BBK developed patient-facing materials targeting participants who had taken part in previous research studies of the same condition. And because patient panels were key to identifying eligible participants, BBK provided data-mining guidance during every step of the recruitment process.