

# Case Study: Optimal Pathways to Commercial Success for Orphan Drug Innovators

## Situational and Market Access Challenges

In preparation for the launch of a novel orphan drug, the manufacturer engaged Boston Healthcare Associates, Inc. (BHA) to develop a market access strategy that would increase the likelihood of separate payment across multiple settings of care.

- Unpredictable and acute condition
- No specific diagnosis code (like other orphan conditions)
- Novel drug administered in Emergency Department
- Policy issues: two midnight rule, transition of care, observation care, comprehensive APCs
- Achieving separate payment
- Demonstrating product value
- Generating supporting evidence
- Obtaining appropriate disease / products codes
- Increasing disease awareness

### Methodology and Approach

- **Health Policy Analysis:** BHA reviewed government (Medicare, Medicaid, VA/DoD, TRICARE) and Private Payer coverage and payment policies
- **Target Product Profile:** BHA created stakeholder-specific primary research materials
- **Primary Research Interviews:** BHA completed interviews with key stakeholders
- **Payer mix and claims analyses:** BHA analyzed key datasets
- **Strategic working sessions:** BHA facilitated multiple working sessions to report findings and refine strategy / tactics

### Key Stakeholder Analysis

- **Physicians:** BHA recruited key opinion leaders to discuss important factors that drive product utilization
- **Policy/Regulatory Agencies:** BHA analyzed policies to understand potential for separate payment in a complex landscape
- **Hospital Admins/P&T Committees:** BHA interviewed key hospital stakeholders to map the product adoption decision-making process
- **Payers:** BHA utilized extensive payer knowledge and contacts to determine likelihood of utilization restrictions and payment
- **Coding/Billing Stakeholders:** BHA verified coding requirements and addressed coding gaps across settings of care

## Key Findings

- Patient transition of care determined payment methodology
- Key drivers of product adoption (e.g., outcomes, safety, unmet need, price) differed across stakeholders
- Given anticipated infrequent use, product expiration and replacement programs outweighed price discounts to purchasers
- Providers indicated the minimum evidence requirement to support adoption, informing the development of evidence generation strategy
- Private Payers may base a drug's payment on individual Payer-Provider contract negotiations or Medicare payment policies

## Deliverables

- Initiated a strategy to obtain separate payment across multiple settings of care
- Developed value communication tools and launch materials for key accounts (e.g., value dossier, billing guide, and formulary kit)
- Completed various coding applications (e.g., ICD-10-CM and ICD-10-PCS, HCPCS)

## Outcome

- Client used Boston Healthcare's analysis to develop and execute their reimbursement strategy

By Thomas F. Goss, PharmD and Kyle Clifton  
Email [info@bostonhealthcare.com](mailto:info@bostonhealthcare.com)