Customized Patient Care through Patient Support Programs for Rare/Orphan Drugs

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Why Orphan Drugs are Different

- Difficult to diagnose
- Population size
- Unique patient and caregiver needs
- Cost
- Reimbursement challenges
- Wait times for Specialist
- Large amount of stakeholders involved to get patient on therapy

There is no clear path to get Orphan Drugs Funded
A Typical Journey of a Patient with a Common Disease

**Hospital-Administered Medication**

1. Discuss Treatment Options
2. Schedule Treatment Date
3. Administer Medication (Nurse)
4. Monitor

Cancer Centre/Hospital*

**Take-Home Medication**

1. Discuss Treatment Options
2. Receive Prescription
3. Pick Up Drug at Pharmacy
4. Administer Medication
5. Monitor

Physician Office

Pharmacy**

Patient’s Home

Patient’s Home
The Patient Journey in Rare Disease

PSP Introduction
- Coverage information is captured
- Services explained

Enrolment in PSP
Demographics, clinical history

Patient set up to receive treatment

Coverage is secured
- Injection/Infusion Services?
- Life time Max
- Copay?

Call – review Coverage decision

SA submissions

Explore Reimbursement coverage

Onboarding

Prescription

Journey to Dx

Rx

Infusio/Injectionn

Continued Care Calls
- Determine status
- Coach patient

Maintenance

Annual Renewals
Why are orphan Drugs Different?

- Different reimbursement routes across Canada. Public reimbursement/Private reimbursement – 50/50
- Traditionally very high cost medication requires additional administrative work, i.e., Special authorization
- Increase in private payer restriction calls for strong private payer strategy
- Small patient populations may have regional bias
The Patient Perspective

The high drug cost, and out-of-pocket component, (co-payment or deductible) can be unmanageable for many patients (especially for Orphan drugs ~hundreds of 000s/year)

Most income-based public plans such as Trillium in Ontario require patient contributions in the form of co-payments/deductibles (~3-5% of household income)

With complex therapies, the primary concerns for patients are access, convenience and the level of customer service

Private plans vary: range from 100% coverage to 50% coverage, though the most commonly seen co-payments are set between 0 and 20%
Case Study: Patient Access Barriers Defined

Figure 1. Factors identified as affecting access to treatment for PKU with applicability to other patients diagnosed through newborn screening.77

- Complex treatment regimen25,34,51,73,91
- Slowly progressing nature of sequelae of non-adherence80,91,92
- Divergence of patient population and their treatment requirements25,34,51,73,91
- Awareness of treatment options and recommendations64,77,80-85,91
- Education of the patient and their families77,80,81,83,87,93,96
- Neurocognitive impairment leading to compromised ability to follow complex treatment regimen and schedule and/or keep appointments73,89,91,93,96,97
- Lack of relationship with healthcare professional80,91,92

Needs for Orphan Rare Disease PSPs

- **High Touch**: Require specific administration or monitoring requirements before and ordering administration.

- **High Support**: Education, counselling, compliance monitoring, and drug information.

- **Special Distribution**: Special handling techniques; and/or require special handling, have special shipping requirements or need complex reconstitution.

- **Customized Patient Assistance**: Clinical, Diet and Lobbying/Financial Support to assist with access to drug (i.e. co-pay assistance).
Customized Patient Support Programs

Nurse Case Manager – One point of contact to deliver quality care at every touch-point

**Enrolment:** Entered in CRM, NCM assigned, patient contacted

**Reimbursement:** Case Manager confirms coverage with the patient and sets up financial assistance

**Infusion Scheduled:** Clinic chosen, Fax to physician, Pre-infusion checklist, Reminder call, Drug order

**Specialty Pharmacy:** Deferral of copay, Drug Order (Direct through Database), Warehouse sends drug to pharmacy, Drug delivered to Clinic

**Infusion:** Nurse confirms Drug is onsite, Pre-infusion assessment by nurse, Medical clarification needed?, Infusion, PIR
Case Manager / Reimbursement Specialist interacts with key stakeholders and customers

- Highly experienced in Orphan/Rare products and motivated by offering best in class patient care
- Experienced in coordinating patients therapy regimens
- The Case Manager role is the one point of contact for the patient and their HCP
- Experience working with Specialist’s offices

Regular and customized updates on patient progress
Pre & Post clinical assessment follow-up
Schedules and coordinates patient infusions

START: Enrolment
Coordinates reimbursement & Co-pay
Extensive Knowledge and experience in Rare Disorders

Case Study 1

Challenge:
Patient was hospitalized with multiple organ failure. The medication was still under review by the Province.

Solution
– Public EAP requested
– Patient Advocacy: Lobby on behalf of patient; media releases; Canada wide petition; add pressure to decision makers; weekly telephone conferences with stakeholders

Outcome
– Provincial compassionate program obtained to start and then successfully gained full approval from the province

Patient is still on therapy!
Challenge:
Patient had a major stroke and hospitalized for over 2 years. Patient is in need of drug to prevent future thrombotic events, hospital refused to pay out of their budget.

Case Study 2

- **Solution**
  - Public/Private
  - Requested that private payer cover medication (even though patient was hospitalized)
  - Concierge (FCM) went to the physician’s office to complete paperwork
  - Provided private payer clinical evidence to support patient stability and improvement

- **Outcome**
  - Gained approval through private payer for a period of one year after demonstrating clinical need
Conclusions

> Rare diseases present unique challenges for:

- Diagnosis
- Testing
- Reimbursement
- Financial assistance
- Ongoing treatment
- Monitoring
Goal: Manufactures should leverage the PSP to demonstrate real world effectiveness value to gain access to payers.

Our ultimate objective is to build a different framework for Orphan Drugs which will shorten the timelines to access.
Thank-you
Capitalize on PSP to generate real world data

Purpose: To decrease reimbursement and commercial timelines

Patient Support Programs — Objectives
- Allow patients to access commercial therapy
- Assist in the navigation of multidisciplinary services
- Coordinate patient logistic services (F/A, Pharmacy, Distribution, Nursing)

Post – Marketing Surveillance and Real World Data - Objectives
- To track outcomes in real world setting
- To illustrate and prove value of drug

Reimbursement – Objectives
- To gain coverage and funding with payers
- To gain coverage on behalf of patients

Clinical Research
- To prove clinical efficacy and safety (Phase I–III)
Where knowledge, reach and partnership shape healthcare delivery.