Navigating Disruptive Launches

Lessons Learned From the Trenches

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Digital Pharma Engage

May 14, 2024

Before we dive in..

A bit about me…

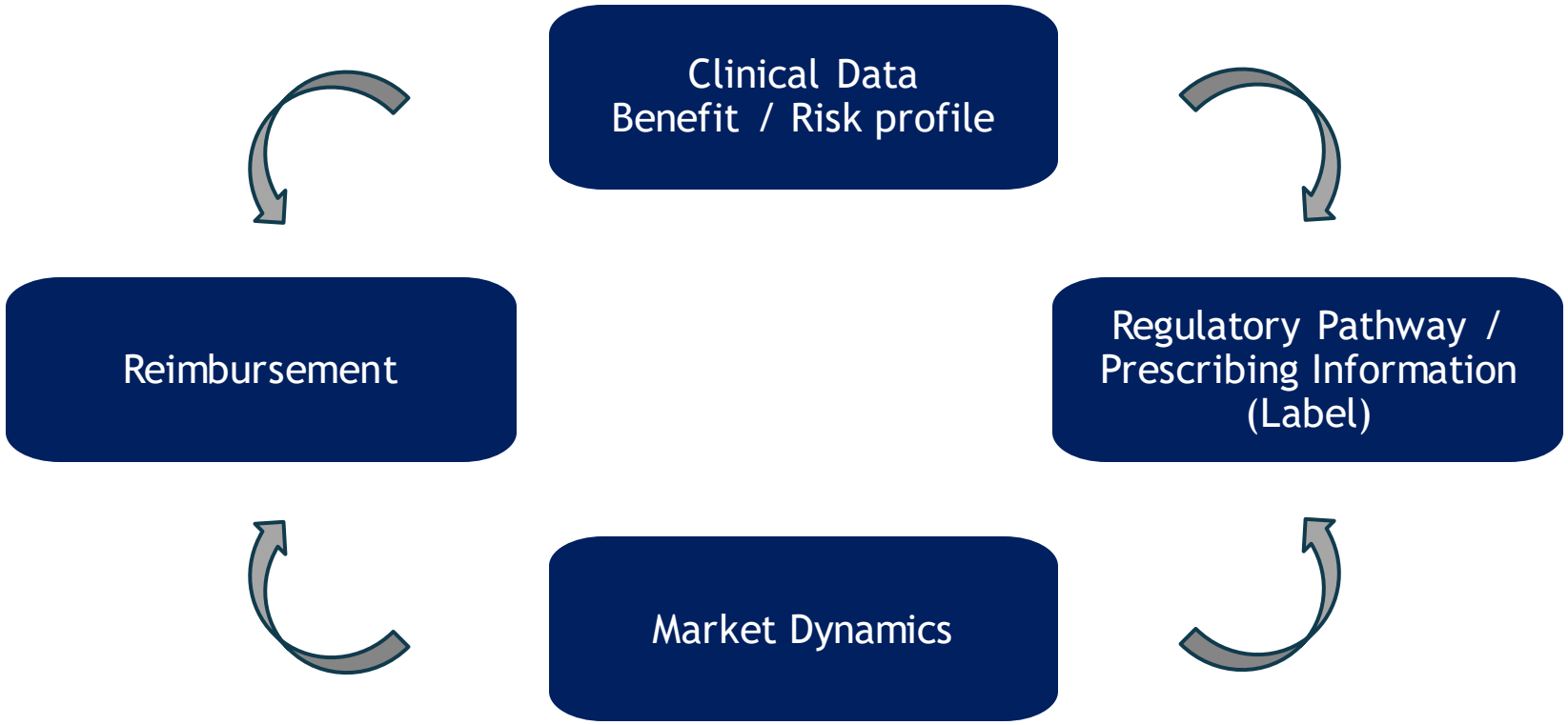






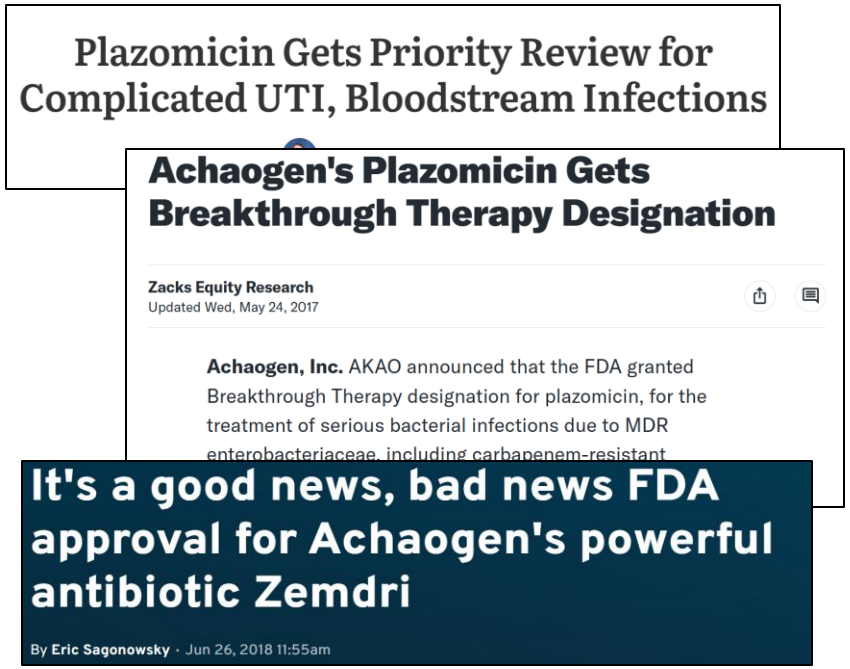


A few key drivers of Commercial success



Gain a clear understanding of clinical data and regulatory pathway

- Developing trusting relationships is key
- Partner closely with Regulatory / Clinical / Development teams
- Identify key success factors for prescribing information (label) and FDA negotiations



Plazomicin Gets Priority Review for Complicated UTI, Bloodstream Infections

Achaogen's Plazomicin Gets Breakthrough Therapy Designation

Zacks Equity Research
Updated Wed, May 24, 2017

Achaogen, Inc. AKAO announced that the FDA granted Breakthrough Therapy designation for plazomicin, for the treatment of serious bacterial infections due to MDR enterobacteriaceae, including carbapenem-resistant

It's a good news, bad news FDA approval for Achaogen's powerful antibiotic Zemdri

By Eric Sagonowsky · Jun 26, 2018 11:55am

----------**INDICATIONS AND USAGE**----------

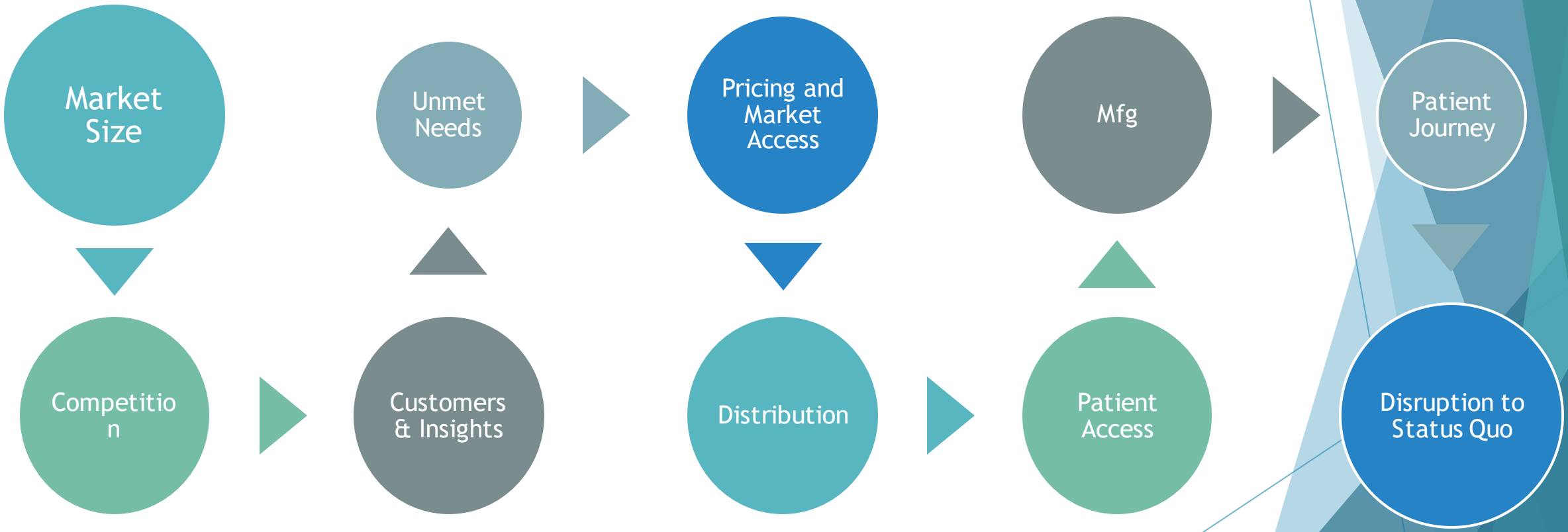
ROCTAVIAN is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with severe hemophilia A (congenital factor VIII deficiency with factor VIII activity < 1 IU/dL) without pre-existing antibodies to adeno-associated virus serotype 5 detected by an FDA-approved

----------**WARNINGS AND PRECAUTIONS**----------

- Infusion-related reactions: Infusion reactions, including hypersensitivity reactions and anaphylaxis, have occurred. Monitor during and for at least 3 hours after ROCTAVIAN administration. If symptoms occur, slow or interrupt administration and give appropriate treatment. Restart infusion at slower rate once symptoms resolve. Discontinue infusion for anaphylaxis. (2.3, 5.1)
- Hepatotoxicity: Monitor alanine aminotransferase (ALT) weekly for at least 26 weeks and institute corticosteroid treatment in response to ALT elevations as required. Continue to monitor ALT until it returns to baseline. Monitor factor VIII activity levels since ALT elevation may be accompanied by a decrease in factor VIII activity. Monitor for and manage adverse reactions from corticosteroid use. (5.2)
- Thromboembolic events: Thromboembolic events may occur in the setting of elevated factor VIII activity above the upper limit of normal (ULN). Factor VIII activity above ULN has been reported following ROCTAVIAN infusion. Evaluate for risk factors for thrombosis including cardiovascular risk factors prior to and after ROCTAVIAN use and advise patients accordingly. (5.3)
- Monitoring laboratory tests: Monitor for factor VIII activity and factor VIII inhibitors. (5.4)
- Malignancy: Monitor for hepatocellular malignancy in patients with risk factors for hepatocellular carcinoma (e.g., hepatitis B or C, non-alcoholic fatty liver disease, chronic alcohol consumption, non-alcoholic steatohepatitis, advanced age). Perform regular liver ultrasound (e.g., annually) and alpha-fetoprotein testing following administration. In the event that any malignancy occurs after treatment with ROCTAVIAN, contact BioMarin Pharmaceutical Inc. at 1-866-906-6100. (5.5)

Source: Medical Professionals Reference, accessed May 2024, https://www.empr.com/home/news/drugs-in-the-pipeline/plazomicin-gets-priority-review-for-complicated-uti-bloodstream-infections/; Yahoo! Finance, accessed May 2024, https://finance.yahoo.com/news/achaogen-apos-plazomicin-gets-breakthrough-150803961.html; Fierce Pharma, accessed May 2024 https://www.fiercepharma.com/achaogen-wins-fda-approval-for-new-antibiotic-zemdri-limitations; ROCTAVIAN prescribing information, accessed Jan 2024

Assessing the market is also critical…



…in order to develop and implement strategies to reach your desired future state

Understand Market

- Customers
- Insights: beliefs & drivers
- Market size
- Competition
- Unmet needs
- Patient journey

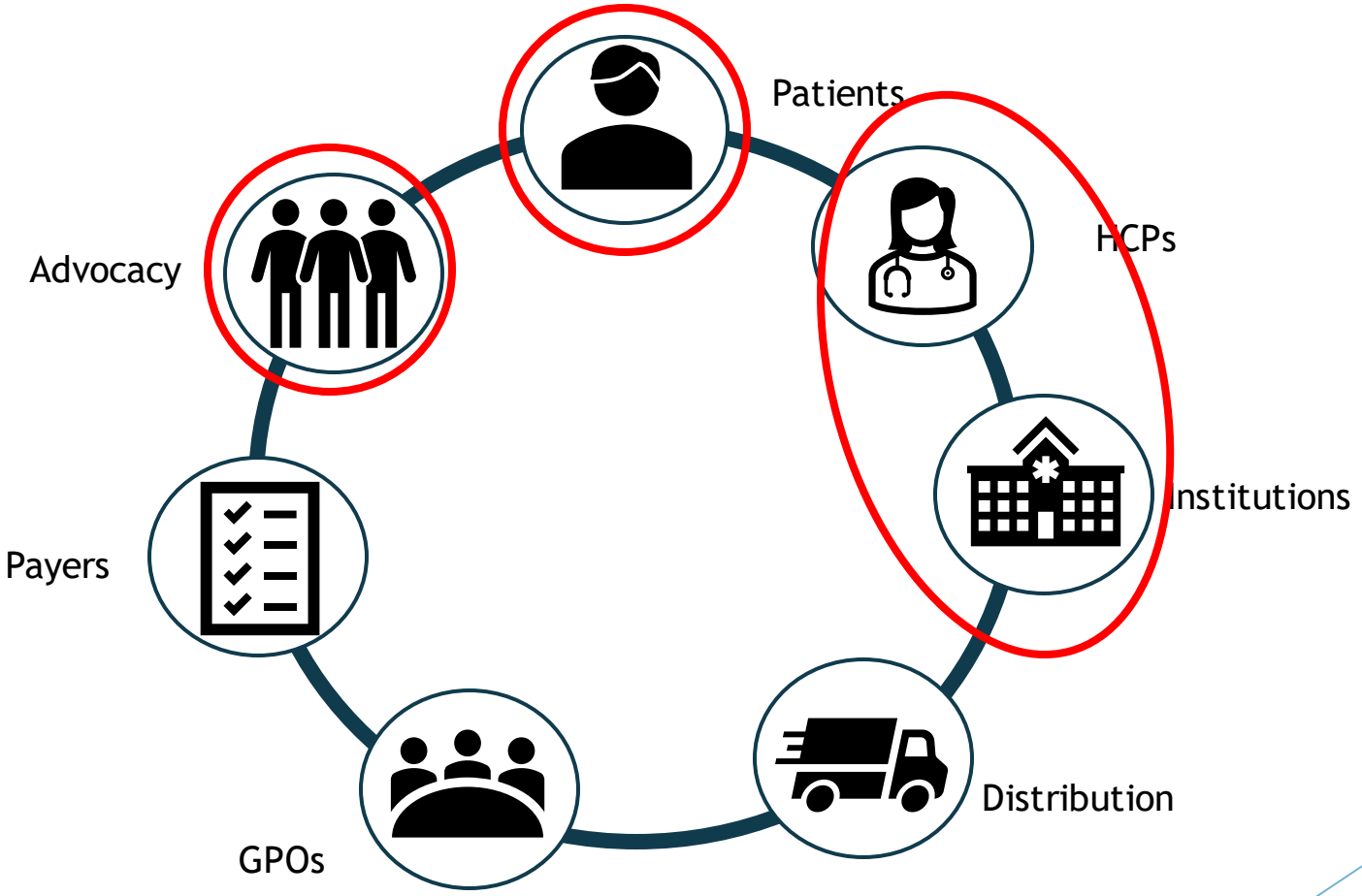
Identify Desired Future State

- Perception of product
- Patient journey
- Needed education
- Critical success factors
- Hurdles to overcome

Develop & Implement Strategies

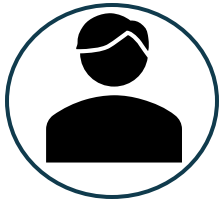
- Compliant pre- and post-launch activities & scope
- Ways to overcome hurdles
- Influencers and levers
- Messaging & tactics

Need to build external relationships early to understand key stakeholders & drivers



- What's changing?
- What's staying the same?

Patients



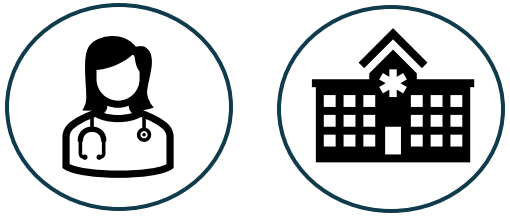
Sample Insights & Questions

- Segmentation
- POV, objections
- Messaging
- Communication channels

Sample Ways to Learn & Build Relationships

- Market research
- Advisory boards
- Patient advocacy groups
- Influencers
- Social media / social listening

HCPs & Institutions



Sample Insights & Questions

- Segmentation
- POV, objections
- Messaging
- Communication channels
- New processes, training, infrastructure

Sample Ways to Learn & Build Relationships

- Market research
- Advisory boards
- Field personnel
- Clinical team insights
- Conferences

Advocacy



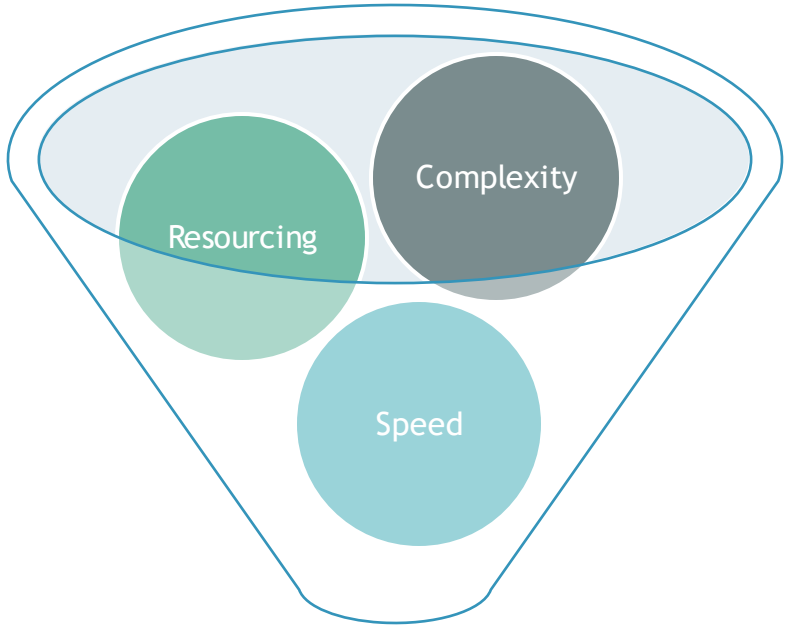
Sample Insights & Questions

- Historical context
- POV, objections
- Patient & caregiver perspective
- Educational needs & delivery methods

Sample Ways to Learn & Build Relationships

- Market research
- Advisory boards
- Field personnel
- Conferences
- 1:1s

Various considerations can impact internal resource buildout



- Insights gathering
- Compliant unbranded disease education
- Strategy & tactic formulation

- Branded strategy and tactic execution
- Market tracking
- Continued insights gathering

Internal team buildout

PDUFA

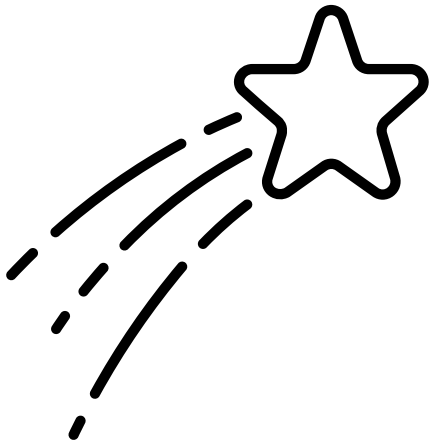
Certain unique aspects of launch may add complexity as well

Companion Diagnostic (CDx)

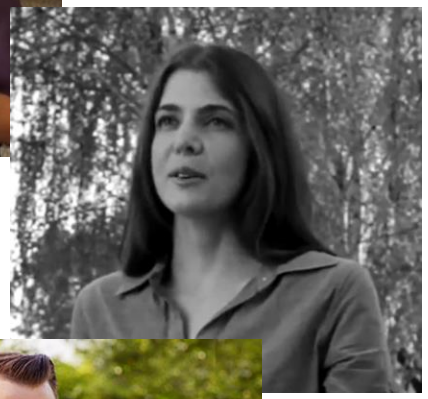


- Reviewed by CDRH
- Approved at the same time as the drug
- Drug label references test; test label references specific drug
- May be developed by third-party labs
- Need to gain alignment
  - Objectives
  - Priorities
  - Commercialization plans

Always with the ultimate goal in mind









Source: https://www.rituxan.com/gpa-mpa/faqs-and-resources/support.html, https://www.korlym.com/patient-advocacy-support/patient-testimonials/#saras-story, https://www.roctavian.com/en-us/roctavian-stories/

THANK YOU!