

Porphyria Targeting Therapies Market, 2021-2030 by Roots Analysis

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/EINPresswire.com/ -- Several novel and innovative therapeutic approaches, both small molecule and biologics, are being currently investigated at various phases of development in order to avoid the beginning of disease-induced attacks and other long-term effects of porphyria

[Roots Analysis](#) has announced the addition of “[Porphyria Targeting Therapies Market, 2021-2030](#)” report to its list of offerings.

Porphyria is a rare disorder that is characterized by excessive accumulation of porphyrin, a compound that aids in the formation of heme (an essential part of hemoglobin that helps carry oxygen in blood). Any anomaly caused by genetic or acquired abnormalities in heme biosynthesis (produced majorly in bone marrow and liver) can result in toxicity. It is worth highlighting that, till date, more than 1,000 mutations that can cause porphyria have been identified. However, prevalence of porphyria still remains unknown. Several treatment options such as gene therapy, proteasome inhibition and pharmacologic chaperones are currently being investigated among various other targeted treatment options.

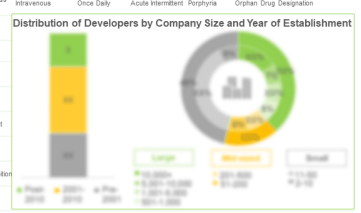
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Example Highlights

List of Drug Candidates^{1, 2, 3}

S. No.	Developer	Drug Candidate	Phase of Development	Drug Class	Mechanism of Action	RoA	Dosing Frequency	Type of Porphyria	Drug Designation
1	Abylam	OPCARB	Approved	Biologic	ALA51 mRNA Inhibitor via RNA Interference	Subcutaneous	Once Monthly	Acute hepatic Porphyria	Breakthrough Therapy Designation, Priority Review Designation, Orphan Drug Designation
3	CLINIVEL	SCENESSE [®]	Approved	Biologic	Melanocortin 1 Receptor (MCL1R) Agonist	Subcutaneous Injekt	NA	Erythropoietic Protoporphria	Orphan Drug Designation
7	ROCHE	Patheonin™	Approved	Biologic	(Delta)-Aminolevulinic Acid Synthetase Inhibition	Intravenous	Once Daily	Acute Intermittent Porphyria	Orphan Drug Designation
8	Novartis	MT-7117	Phase II	Small Molecule	Melanocortin 1 Receptor (MCL1R) Agonist				
8	Novartis	Coledil	Phase II / III	Small Molecule	Reduction in lipid level				
8	Harvon®	Harvon®	Phase II	Small Molecule	HCV NS5A / HCV NS5B Inhibitor				
9	diagnostic	Eltopirin	Phase I	Small Molecule	GlyT1 Selected Inhibitor				
10	CYCLE PHARMACEUTICALS	Unnamed	Phase I	Small Molecule	Dopaminergic Receptors Antagonist				
11	PALATIN	Unnamed	Preclinical	Biologic	NA				
12	Atlas	AMP-L2.7.07	Preclinical	Small Molecule	Uroporphyrinogen III synthase inhibitor				
13	moderna	Unnamed	Preclinical	Biologic	NA				

Distribution of Developers by Company Size and Year of Establishment




Abbreviations: RoA, Route of Administration
 Note 1: Information in this report (and specifically this table / figure) has been identified from publicly available sources on a best-effort basis. However, we realize that some of the data points may not be publicly available and, as such, may have been overlooked in our analysis. If you'd like to notify us of these gaps, please send an email to support@rootsanalysis.com
 Note 2: The products listed in the table have been arranged in decreasing order of phase of development

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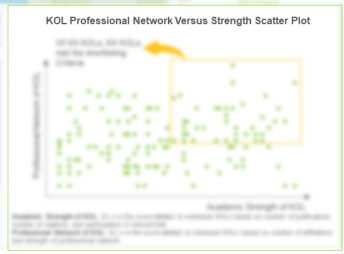
Porphyria: Pipeline Review, Developer Landscape and Competitive Insights

Example Highlights

Porphyria¹
 Mapping Key Opinion Leaders (KOLs)¹



KOL Professional Network Versus Strength Scatter Plot



Note 1: Complete list of KOLs that we came across during the study, is available on request

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Porphyria: Pipeline Review, Developer Landscape

<https://www.rootsanalysis.com/reports/porphyria-pipeline-review.html>.

Key Market Insights

15+ therapies have been / are being developed for the treatment of different types of porphyria. More than 70% of the aforementioned candidates are currently under clinical evaluation. Further, three therapies, namely Panhematin™, GIVLAARI® and SCENESSE®, have already been approved for the treatment of different types of porphyria.

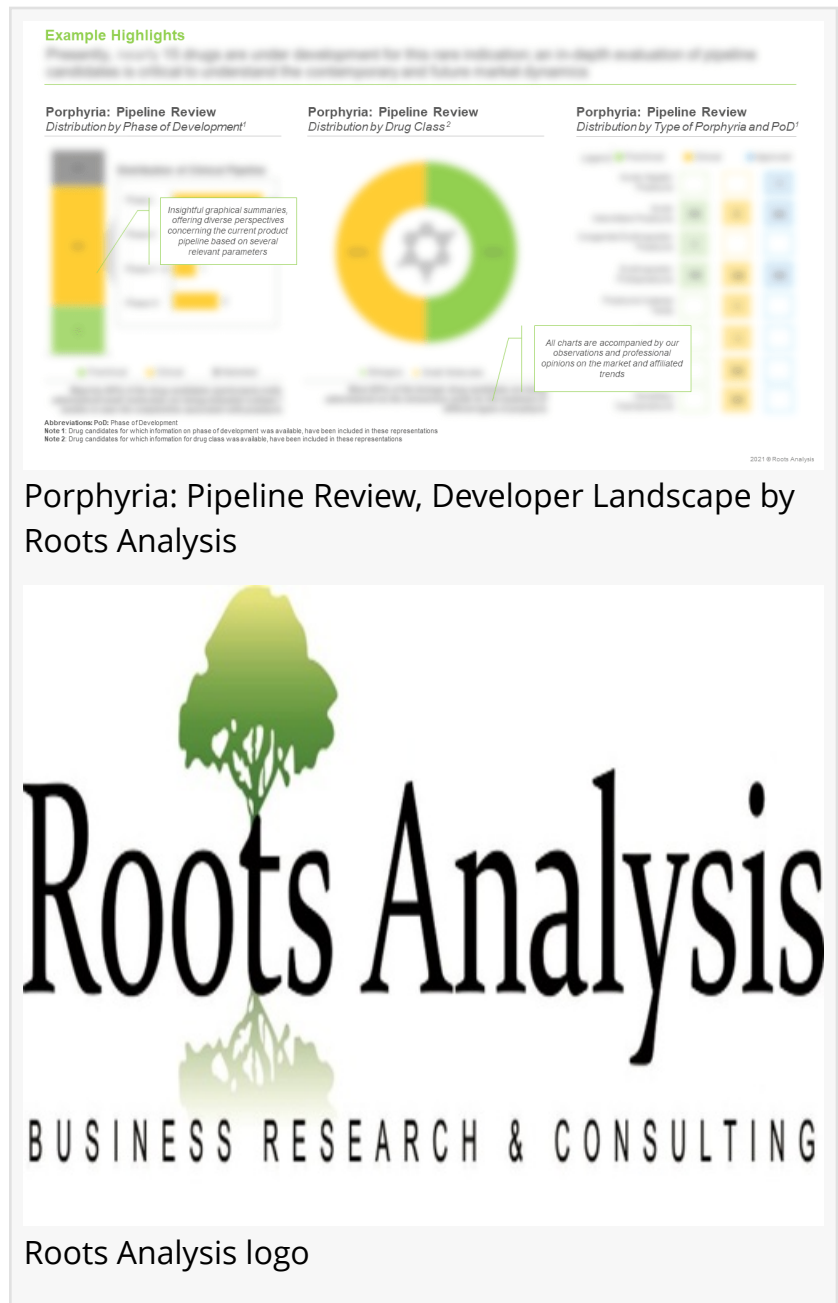
Around 50% of the therapeutics are being developed as biologics. Majority (over 65%) of the abovementioned biologic drugs have been / are being designed for administration via the intravenous route. Furthermore, majority of the drugs (37%) have been / are being targeting acute intermittent porphyria.

Over 30% of the therapies have been / are being developed for erythropoietic protoporphyria.

More than 65% the abovementioned therapies are currently being evaluated in clinical phases. Further, around 60% of the aforementioned therapy candidates are being developed as small molecules.

More than 45% of the players evaluating therapies for porphyria are small companies. North America has emerged as a key hub for the development of porphyria therapies, featuring the presence of 65% developers. The developer landscape is further dominated by players that have been established between 2001-2010, representing around 45% of the total number of stakeholders.

A number of clinical trials evaluating therapies for porphyria, have been registered. Majority of the clinical studies have been completed. More than 30% of the overall trials are phase I studies. Further, it is worth noting that, most of the trials (~ 60%) focused on porphyria.



therapies were registered post-2010.

Partnership activity in this field has increased at a CAGR of 9.6%, between 2018 and 2020. More than 70% of the reported deals were established post-2018, with the maximum activity being reported in 2019 and 2020. Majority of the instances captured in the report were product distribution / commercialization agreements (~45%).

380+ articles have been published related to porphyria, since January 2018. Close to 20% publications mentioned in the report were focused on the assessment of therapeutics that have been / are being developed for the treatment of erythropoietic protoporphyria. Example of prominent journals include (in decreasing order of number of publications) Molecular Genetics, Orphanet Journal of Rare Diseases, British Journal of Dermatology and Molecular Genetics, and Metabolism Reports.

Around 15 eminent individuals were identified as key opinion leaders (KOLs) in this domain. More than 65% of these KOLs were observed to be associated with organizations based in US, followed by those affiliated to institutes in Spain (20%) and South Africa (7%). Further, over 65% of the KOLs are currently affiliated to academic institutes, such as schools and universities.

North America is anticipated to capture over 60% of the global market share in 2030. In 2030, more than 50% of the market revenues are expected to be generated from sales of therapeutics intended for the treatment of erythropoietic protoporphyria and porphyria cutanea tarda. Further, therapies designed for oral route of administration are expected to occupy a larger share (51%) of the overall market, in the foreseen future.

To request a sample copy / brochure of this report, please visit this link.

Key Questions Answered

- What are the prevalent R&D trends related to Porphyria?
- What are the key challenges faced by stakeholders engaged in this domain?
- What are the principal therapies developed by the companies in this domain?
- Who are the leading industry and non-industry players in this market?
- What are the key geographies where research on porphyria is actively being conducted?
- Who are the key investors in this domain?
- Who are the key opinion leaders / experts in this field?
- What kind of partnership models are commonly adopted by industry stakeholders?
- What are the factors that are likely to influence the evolution of this upcoming market?
- How is the current and future market opportunity likely to be distributed across key market segments?

The financial opportunity within the porphyria therapies market has been analyzed across the following segments:

- Drug
- GIVLAARI[®]
- Banhematin[®]
- SCENESSE[®]
- MT-7117
- Colestid
- HARVONI[®]

- Type of Porphyria
- Acute Hepatic Porphyria
- Acute Intermittent Porphyria
- Erythropoietic Protoporphyrria
- Hereditary Coproporphyrria
- Porphyria Cutanea Tarda
- Variegata Porphyria
- X-Linked Porphyria

- Route of Administration
- Oral
- Intravenous
- Subcutaneous

- Key Geographical Regions
- North America
- Europe
- Asia-Pacific

The research includes profiles of key players (listed below); each profile features a brief overview of company, pipeline details, recent developments (including collaborations and expansions) and an informed future outlook.

- Agios Pharmaceutical
- Anylam Pharmaceuticals
- Clinuvel Pharmaceuticals
- Disc Medicine
- Mitsubishi Tanabe Pharma
- Moderna Therapeutics
- Balatin Technologies
- Recordati Rare Diseases

For additional details, please visit <https://www.rootsanalysis.com/reports/porphyria-pipeline-review.html> or email sales@rootsanalysis.com

You may also be interested in the following titles:

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