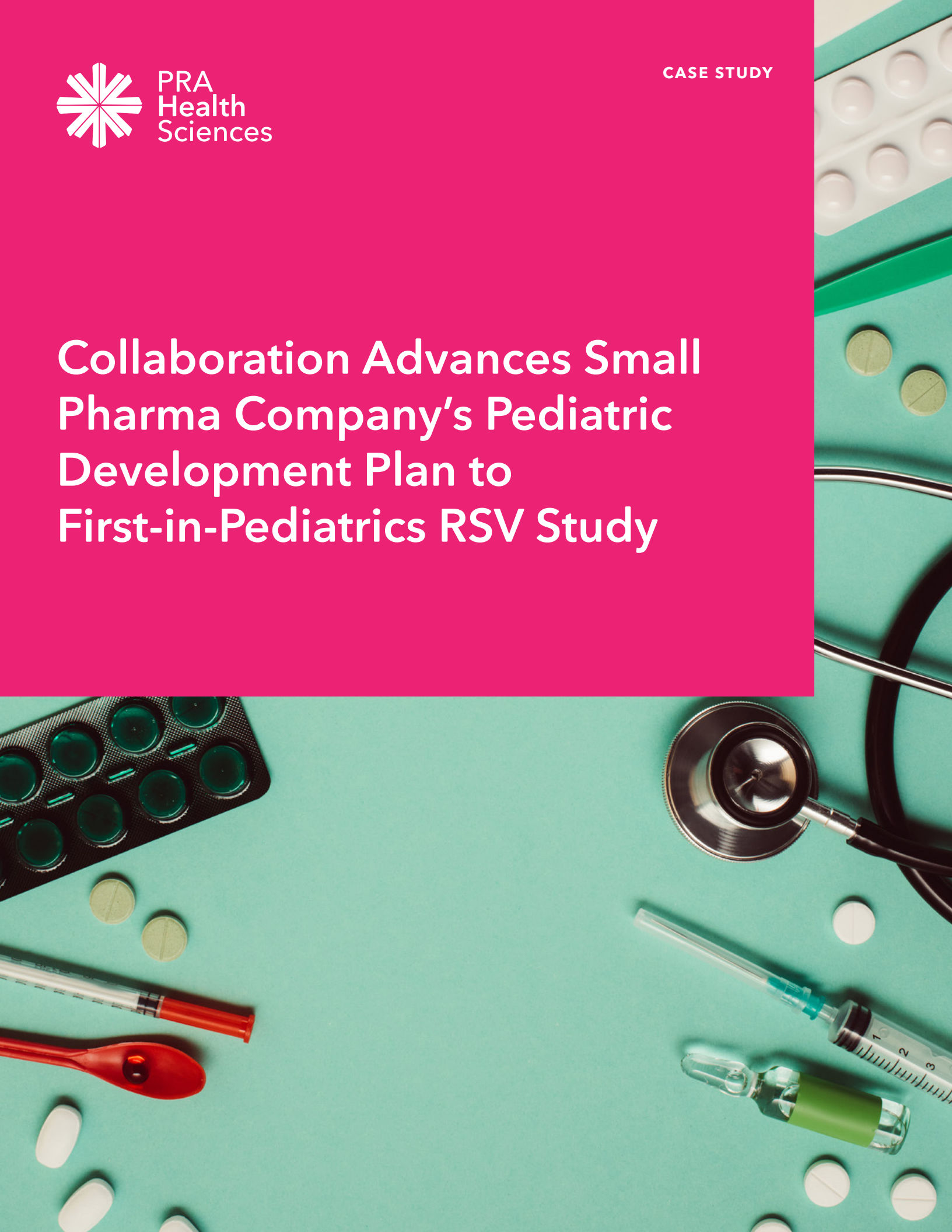
PRA Health Sciences

CASE STUDY

Collaboration Advances Small Pharma Company's Pediatric Development Plan to First-in-Pediatrics RSV Study



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STUDY DESCRIPTION

A Phase II, randomized, double-blind, placebo-controlled, 2-part study to evaluate the safety, tolerability, pharmacokinetics, clinical response, and antiviral activity of orally administered study drug regimens in hospitalized or non-hospitalized infants and children aged 28 days to 24 months infected with RSV (respiratory syncytial virus).

Primary Endpoints

- To evaluate the pharmacokinetics of the study drug
- To evaluate the antiviral activity of the study drug

PRA Services

Center for Global Drug Development - strategic protocol development

Center for Pediatric Clinical Development - consulting for pediatric drug development

Pediatric Site Network - site relationship development

Global Regulatory Affairs

Clinical Pharmacology

Full-scope clinical trial conduct

Indication

Respiratory syncytial virus

Drug Class

Antiviral

Study Phase

Phase Ib/IIa

Business Segments

Product Registration
Scientific and Medical Affairs

Study Duration
24 months

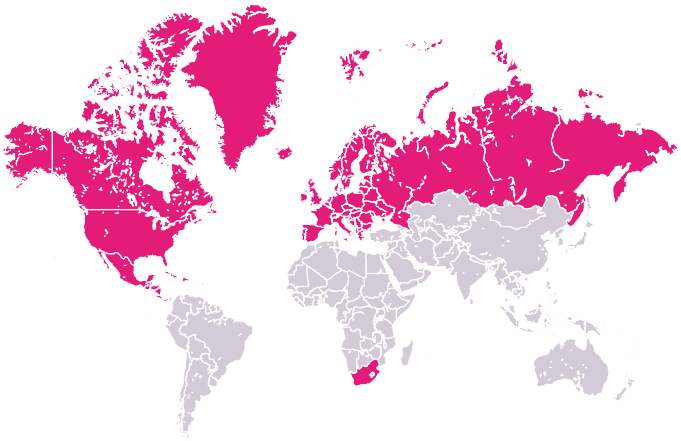
No. of Clinical Sites
The number of sites is in development; we anticipate 40-50 sites globally.

Patient Population
88 hospitalized or non-hospitalized infants and children, aged 28 days to 24 months, with an RSV-associated respiratory tract infection, and who test positive for RSV based on an approved diagnostic assay.

Regions

We anticipate sites in the following regions:

North America
Latin America
Western Europe
Central Europe
Eastern Europe
South Africa



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Situation

The client, a small to midsize biopharma company, is developing a small molecule for the treatment of RSV in adult and pediatric patients. RSV is a common and very contagious virus that infects the respiratory tract of most young children, and although rarely serious, the disease can lead to hospitalization and even death.

The client contracted PRA to provide scientific advice on a pre-clinical study design to support a first-in-pediatrics study. The client also sought advice and assistance developing a pediatric clinical strategy and design for a proposed Phase I study in RSV. The initial plan was to discuss only pre-clinical data and a juvenile toxicology study with health authorities. However, as PRA is familiar working with small biotech, and as we anticipated the challenges a small to midsize biopharma company would experience—as well as the unique support required for this study—we advised the client to expand the scope and consider an adaptive Phase Ia/IIb design instead of the originally planned standard Phase I design.

Led by our Center for Pediatric Clinical Development (CPCD), Center for Global Drug Development (CGDD), and Global Regulatory Affairs (GRA) teams, PRA provided therapeutic, pediatric, and clinical development expertise, and acted as the project management, therapeutic, and regulatory lead. We developed briefing books for an FDA (US Food and Drug Administration) type B meeting and a brief for the CHMP (Committee for Medicinal Products for Human Use). PRA attended teleconferences with the FDA and the CHMP and provided therapeutic and pediatric expertise. Feedback from the health authorities was analyzed and PRA helped the client adjust their strategy. PRA was able to minimize risk for the client, accelerate overall timelines for development, and obtain additional insights for planning future studies. Based on the trust built between PRA and the client and the positive results garnered in the initial contract, the client awarded PRA a non-compete, full-service contract to finalize the design and begin operations on their Phase IIa study, resulting in a successful partnership for both PRA and the company.

Challenges & Solutions

Some challenges experienced and solutions developed for the initial contract included:

Challenge No. 1:

While the client is experienced in viral infection models, they had limited experience in RSV and almost none in pediatric drug development. The client also had limited experience obtaining scientific advice from health authorities.

Solution No.1:

As PRA's lead physician has years of experience in pediatric RSV, they provided therapeutic expertise. We helped the client develop their pediatric clinical strategy; this included co-authoring briefs, developing their first-in-pediatrics clinical study, submitting the briefs for HA advice, attending health authority interactions, and adapting the program based on feedback.

Challenge No. 2:

The initial client proposal required agency advice on only a limited set of pre-clinical questions.

Solution No. 2:

PRA felt this initial strategy was too basic and would not fully mitigate the risk for the pediatric clinical strategy prior to initiating the first-in-pediatric program. PRA recommended that the client increase the scope of the advice and suggested questions to de-risk the program; the client accepted our recommendations and contracted PRA to provide therapeutic and pediatric expertise and coordinate submission to the authorities on their behalf.

Challenge No. 3:

The client hired new key personnel early in the program. Unfortunately, the new clinical leads had limited experience in the therapeutic area and none working with pediatric patients.

Solution No. 3:

Because of our experience managing pediatric clinical programs, the CPCD team was able to provide insightful, focused advice. The therapeutic expert for the program applied their in-depth background knowledge of the indication to advise.

We leveraged relationships developed through the PRA Pediatric Site Network (PSN), our in-house strategic initiative with key pediatric centers of excellence, to introduce the client to key opinion leaders (KOLs) and increase the client's trust in our ability to deliver. The ability to swiftly obtain advice and clinical expertise from KOLs who treat these patients and have significant research experience in RSV allowed us to develop a scientifically sound protocol tailored to pediatrics in this indication.

Challenge No. 4:

PRA was asked to provide an RFP for an adult program in RSV. PRA was not awarded the adult challenge portion of this study. Data from the adult RSV challenge study is ongoing. As a pediatric plan draws from results of studies in adults, it was challenging to define considerations for the pediatric program without the adult data.

Solution No. 4:

Although the client chose not to use PRA's services, our CPCD, CGDD, and regulatory teams did convince them that our expertise and strategy were accurate and would provide the best route to a successful early pediatric program.

Results

With CGDD, CPCD, and GRAL leadership, the client delivered 2 briefing documents on time and on budget and had successful teleconference interactions with the FDA and the CHMP. The client was extremely pleased with PRA's performance and praised the team. The client expanded the scope of the contract with PRA to include:

- Chemistry, manufacturing, and control (CMC) related inquiries.
- A first-in-pediatrics study design in which a director of drug development and CPCD assisted in developing a draft study synopsis that included a plan to initiate the study design in the US and EU ahead of schedule.
- A modified study design to increase the time to Phase II. This included removal of a portion of the Phase I work and creation of an adaptive Phase Ib/IIa study design.
- PRA introduced the client to KOLs for ongoing research in RSV.

Based on our performance on the initial contract, the client awarded PRA the Phase Ib/IIa study under a non-competitive bid. Startup work is underway. The CGDD and CPCD are providing consulting services and reviewing both the synopsis and full protocol. In addition, the PSN is engaged with the program—the first site to qualify for the study was part of the PSN and one of the KOLs we consulted—and the director of drug development will act as therapeutic expert (with assistance from the CPCD). The study is on track for initiation in Q4 2020 and is expected to run for 24 months.