

# China Orphan Drugs Emergency Aid Project During COVID-19 Action Report

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### Project Overview

The COVID-19 pandemic broke out in China at the end of 2019. It started from Hubei Province and quickly spread across the country. The Chinese government proactively took measures to fight against the pandemic, which including the lockdown of Wuhan from January 23th to April 8th, 2020, a series of restrictions on traveling, and redirection of medical resources and supplies. On the one hand, these measures have prevented the spread of COVID-19. On the other hand, the rare disease community faced an even more challenging situation of accessing treatments and healthcare.

In January 2020, the Illness Challenge Foundation (ICF) received a call for help from the organization of Tuberous Sclerosis(TS) patients, expressing that their community is in an emergency of drug shortage. Because China's medical administration has not approved some methods of treatment for TS, Chinese patients with TS have to buy drugs overseas. Affected by the COVID-19 crisis, international travel and logistics have been restricted. In this case, TS patients in China could not obtain the necessary drugs through previous channels. The drug interruption may cause relapse, may even be life-threatening. This situation did not only occur in the TS group. Just from the end of January to early February this year, the ICF received more calls for help from different rare disease organizations.

On February 11, 2020, the ICF and China Alliance of Rare Diseases (CHARD)<sup>1</sup> formed a joint task force and launched the "China Orphan Drugs Emergency Aid Project During COVID-19". Both the task force and the Project were active for 3 months (from February 11th to April 10th, 2020), which were the worst period of the pandemic in China and when rare disease patients were most in need.

During these three months, the task force liaised with all parties, and responded to the strongest needs from the rare disease community. In particular, the task force provided solutions to 382 rare disease patients who were endangered by drug shortage.

Moreover, to resolve the issues of parent-child communication and lacking of personal protective equipment during COVID-19, the task force distributed the board game "Para

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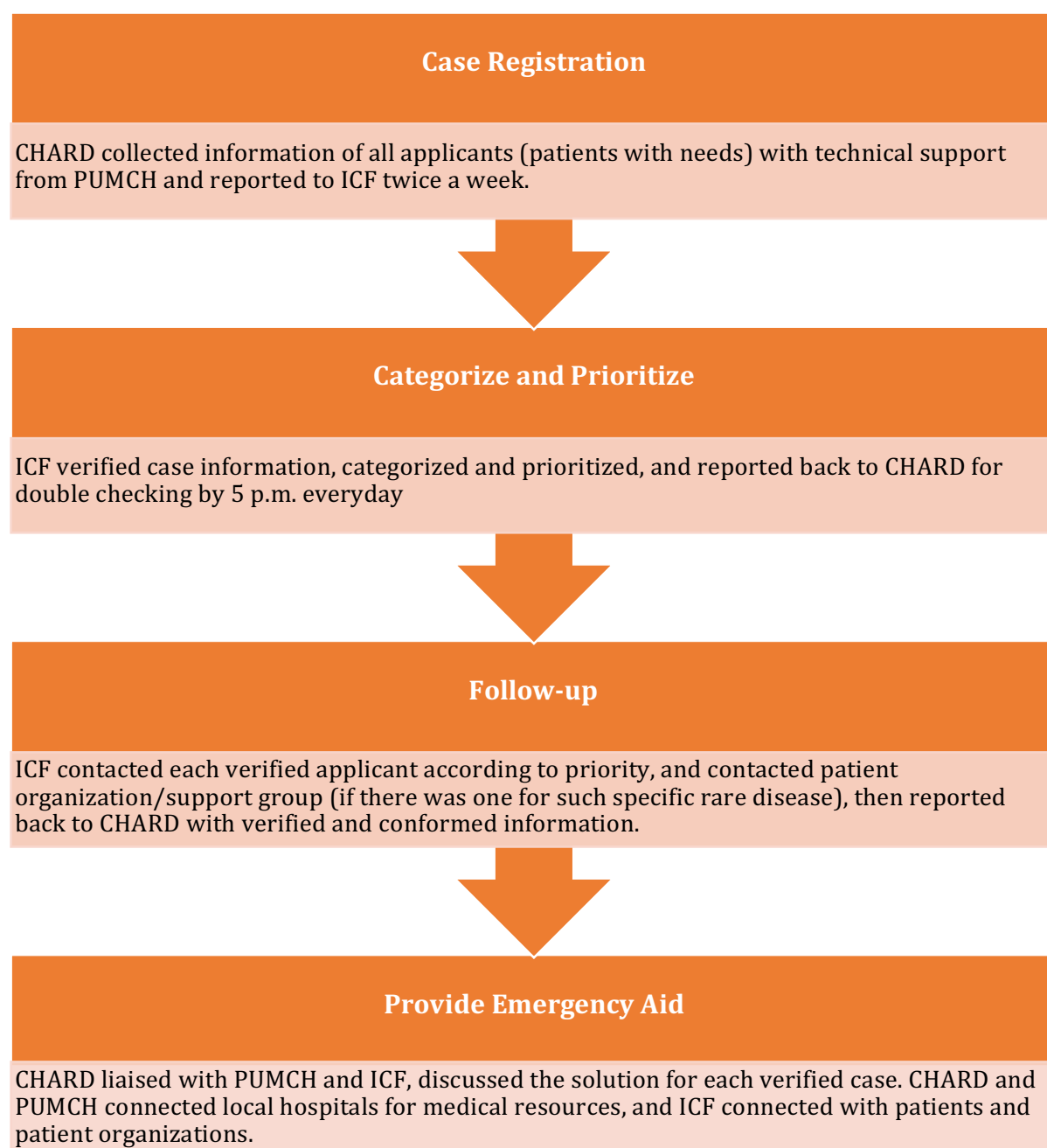
<sup>1</sup> The China Alliance for Rare Diseases (CHARD) is approved by the National Health Commission; led by Peking Union Medical College Hospital (PUMCH), China Pharmaceutical Innovation and Research Development Association (PhIRDA), China Hospital Association(CHA), Chinese Research Hospital Association (CRHA); is composed of medical institutions, universities, scientific research institutes, and industry; is a national voluntary, non-profit, cooperative platform



Ti Baby”<sup>2</sup> to 152 families with rare diseases, as well as 1390 facial masks to 156 families and 5 rare diseases organizations in total.

## Project Details

### Project Procedure



The task force was composed of the staff of CHARD and the ICF, with the division of

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<sup>2</sup> “Para Ti Baby” is a, interactive family board game developed by the ICF for children with rare diseases aged 4-9, to encourage child-parent communication and to reduce psychological stress of the family. For more information: [https://mp.weixin.qq.com/s/drOfwT3JyX\\_cIgRbMH0o8g](https://mp.weixin.qq.com/s/drOfwT3JyX_cIgRbMH0o8g)



labor, timely communication, and orderly connection. CHARD staff designed and implemented an online registration to gather patients' basic information, while the ICF disseminated the project information in patient community.

After the project was started, Since the project started, CHARD exported patients' information on a daily basis, and the ICF verified the patients' information and collected feedback. Each patient's information was reviewed. If there was a patient organization/support group for the specific disease, ICF would confirm the urgency of the needs and learn the current situation of the disease as a whole, such as the reasons causing drug shortage, and whether the organization/support group had response measures. Whereafter, ICF contacted the patients to verify and confirm their needs. Telephone interviews were carried out in terms of the patients' conditions, reasons causing drug shortage, the hospitals and doctors they normally visited, the types and specifications of their drugs, how long they could last with remaining drugs, how serious drug interruption would affect them and if it was life-threatening, and the cost of the drugs (reimbursement/out-of-pocket ratio). Then the ICF categorized and prioritized according to the urgency of needs, listed the names of companies manufactured/supplied the needed drugs, and the number of patients in each specific disease. The most urgent cases were marked individually, followed up closely, and resolved within 1 working day.

CHARD connected with hospitals and companies for solutions. CHARD carefully analyzed the underlying causes of each drug shortage case: was it due to logistics interruption (no international deliveries, the longer time for deliveries, etc.), the decrease of hospitals and medical professionals (medical resources redirected to infected patients, or to severely affected areas), the fact that the patient was in a severely affected area (Hubei Province), or inability to pay (unable to comply with the reimbursement procedures, job loss due to the pandemic, etc.). Then, CHARD provided targeted solutions for each case.

For patients who were not able to travel for hospital visits (Scleroderma, Propionic Acidemia, Acromegaly, etc.), the task force contacted hospitals [including Perking Union Medical College Hospital (Beijing) and the First People's Hospital of Jinzhou (Hubei Province)] to coordinate prescriptions. Meanwhile, the task force actively communicated with local drug supply channels and provided patients with information on local access to treatments.

For patients who were in severely affected areas and on lockdown, the task force contacted DTP pharmacies. If needed drugs were in stock, the task force coordinated with logistics companies for delivery; If they were out of stock, the task force contacted and coordinated with both drug suppliers and logistics companies for solutions. For example, in the cases of patients with Angelman Syndrome in Hubei Province, DTP pharmacies sent the life-saving medicines to three cities in Hubei Province by express delivery at the worst times.



For patients who were unable to comply with the reimbursement procedures due to the pandemic, the task force contacted hospitals and medical professionals, coordinated to prescribe long term repeat prescriptions for the patients to reduce hospital visits. The task force also gathered and disseminated the reimbursement coverage information of local hospitals and pharmacies, and monitored their drug storage. In the cases of Acromegaly, CHARD coordinated with the drug suppliers, who shared information on their national sales network and the reimbursement coverage for hospitals nationwide to Acromegaly patient organization so that the patient organization could help their members to gain access to the drugs during COVID-19.

The task force prioritized the most life-threatening cases, followed up with hospitals, drug companies, pharmacies, and patient organizations/support groups to monitor the progress and outcome. After that, the task force revisited each patient to ensure their emergency was coped with.

Through multiple channels, the task force coordinated with multiple parties, including pharmaceutical companies, commercial companies, DTP pharmacies, industry/academic associations, consulting firms, etc.

During the implementation of the project, the task force also discovered that many patients and families faced other difficulties in the pandemic, such as the shortage of personal protection equipment and parent-child communication barriers. In response to these problems, the task force distributed 1,390 facial masks to 156 families with rare diseases and five rare disease organizations. Meanwhile, 152 rare disease families also received the “Para Ti Baby” board games from the task force.

## **Project Outcomes**

- Provided solutions to 382 patients with rare diseases faced with drug shortage, and published information on treatment accessibility and medical insurance, benefiting more patients with various needs.
- Distributed the board game “Para Ti Baby” to 152 families with rare diseases, and distributed 1,390 facial masks to 156 families and five rare disease organizations to reduce psychological stress and chances of infection.
- Eleven patient organizations, three hospitals, dozens of companies, industry/academic associations, and DTP pharmacies were linked, among which no less than 30 interested parties in total.
- Five media outlets including CCTV, Beijing News, Xinhua Net, Beijing News Broadcasting, and Disability in China reported on the Project in detail.



## Notable Cases

### Case A: Getting Medications for Patients with Scleroderma.

Fu Su Pian, Fu Song Pian, and Fu Kang Pian were three essential medications that patients with Scleroderma needed to take continuously. The most used drug, Fu Su Pian, was compounded and hand-made by Peking Union Medical College Hospital (PUMCH). Patients from all over the country came to the hospital every three months for their routine follow-ups and repeated prescriptions. However, during COVID-19, traffic controls, travel restrictions, combined with the fear of cross-infection in the hospitals, made it very challenging for patients to go to PUMCH and get their prescriptions. After the ICF received several calls for help from patients with Scleroderma, the task force started to work. The ICF verified the needs of the Chinese Organization for Scleroderma. Meanwhile, CHARD reached out to PUMCH, seeking help from Dr. Zhang Tao, the doctor most mentioned by Scleroderma patients. CHARD asked Dr. Zhang Tao to confirm the patients' medical records, verify their needs, and issue prescriptions remotely. Then the task force assisted in delivering the drugs to patients. During this period, CHARD coordinated with PUMCH's Dermatology Department, Outpatient Department, and Hospital Pharmacy to set up a Green Passage (expedited services) for patients with Scleroderma. Over 150 patients with Scleroderma received their medications in 2 batches.

### Case B: The Story of Feng Qinfen.

Feng, a patient with Erdheim-Chester Disease (ECD), a rare type of Non-Langerhans' cell histiocytosis (also known as lipogranulomatosis), was in her sixties and had suffered a lot. Like many other patients with rare diseases who require long-term continuous treatments, Feng needed recombinant human interferon  $\alpha 2a$  injection every other day. Feng visited PUMCH regularly for her treatments, even though she lived in Nanjing, Jiangsu Province, which was hundreds of miles away from the hospital that had the capacity of treating ECD. It was time-consuming and expensive for her and her family to go to Beijing to get the treatments, as long as she could get the treatments for her rare disease, all efforts were worthwhile.

When the COVID-19 hit the whole country during the Chinese Spring Festival holiday season, traffic restrictions were issued and cities were closed off. Feng's medicines from her last prescription were coming short. Each day, Feng and her family crossed their fingers that the pandemic would end soon, which would make the traveling to Beijing possible. On the contrary, the situation was getting worse. Feng's daughter went to all local pharmacies and hospitals but found no recombinant human interferon  $\alpha 2a$  injections in stock.

In the moment of being panic and desperate, Feng's daughter saw the news of the

Project. She applied for her mother, was interviewed by the task force, and waited anxiously for a solution. Just in a day or two, the task force called back to Feng's daughter with the good news that there were recombinant human interferon  $\alpha 2a$  injections supplies in Nanjing, Jiangsu Province. However, it turned out to be a different dosage form, which was much to the disappointment of Feng's daughter. She could not do anything but to wait for further information. Soon after, CHARD confirmed that there were the same treatments that Feng used in Nantong (about 250 kilometers away from Nanjing, where Feng lived), Jiangsu Province. The ICF confirmed with Feng's daughter that she was able to travel to Nantong. Then the pharmaceutical company which manufactured the treatments made sure the treatments would be delivered to a specific pharmacy timely for Feng's daughter to pick up. At last, all settled satisfactorily.

## Conclusion and Reflection

1. The complexity of the rare disease was further revealed through the pandemic, the solution of which must be found by the collaboration of all parties.

The journey to solve rare disease problems had always been difficult. It's a complicated systematic issue. During such a period of the pandemic, all aspects of complexity were exposed more clearly. For example, although the task force contacted drug companies and suppliers, local pharmacies, and online medical service providers, needed treatments for patients with Hemophilia in Hubei Province couldn't reach them. Cold Chain Logistics for the delivery of the treatments wasn't possible. It was against lockdown policies and too risky for patients to go to hospitals or pharmacies. Every party involved had different concerns and interests, which was both true and frustrating. But, no matter what, the complexity of the rare disease problem made it more clear that the only way to tackle this problem was to bring in all stakeholders and collaborate.

2. The different perspectives of all parties led to different actions.

The industry was not as motivated throughout the Project with their interests being concerned. Patient organizations/support groups had different expectations. During the Project, all parties worked closely to fight against this emergency, which was also an opportunity of re-thinking expectations, values, and boundaries. As a patient organization with the vision of knitting all parties together and empowering the patient community, ICF should re-evaluate its role in solving rare disease problems and which way to lead its actions and partners. For example, ICF shared the availability and information of a specific drug with patient organizations and the patient community. However, some organizations and patients considered the information as medical advice, which they should not be used as medical advice. They expected to rely more on





the ICF to make healthcare decisions, which could be indeed risky and needed to be handled with caution. What the ICF provided should only be information on drug availability, while medical advice should be made by professionals. Patient organizations and patients should be more autonomous and responsible for their community's or their healthcare. Generally, patients were more knowledgeable about their diseases. This was a discussion worth to be brought up by the ICF in the hopes that patient organizations/support groups could be empowered on how to filter/verify/deliver information to their community.

### 3. New learning of patient organizations

The ICF worked with various rare disease-specific patient organizations. The development of these patient organizations was very different. Some were registered non-profit organizations with several full-time staff (developed organizations). Some were support groups operated with a few volunteers (developing organizations). Developed organizations had strategic planning, projects, operating team, funding, and were able to mobilize more and wider community. However, during the Project, the ICF gained more insight into developing organizations. Even though some of the organizations only operated by volunteers, they still managed to reach out to their community and resolve their urgent needs. For example, the Chinese Organization for Scleroderma was a young, growing organization, which only had one full-time staff (founder, a scleroderma patient). During the Project, the Chinese Organization for Scleroderma managed to mobilize their patients as volunteers to divide labor and cooperate to respond to the emerging needs. It did not necessarily have to be a registered organization with full-time staff to do things right. As long as you were determined, devoted, and cared for the real needs of the community, one person could do a lot. In the end, not only the drug shortage for individual patients were resolved, but also a Green Passage which would benefit the whole community was set up, with support from the doctors, hospital, and CHARD. It was enlightening for the ICF on future cooperation with patient organizations: what characters of the organizations/leaders should be valued most when taking action.

For future actions and projects, the ICF should engage more patient organizations, establish collaboration with more parties besides CHARD, and create more opportunities for communicating with the patients for all ICF staff, to improve empathy and service quality. With these experiences and thoughts, the Chinese rare disease community should move forward in solidarity.

### Pictures





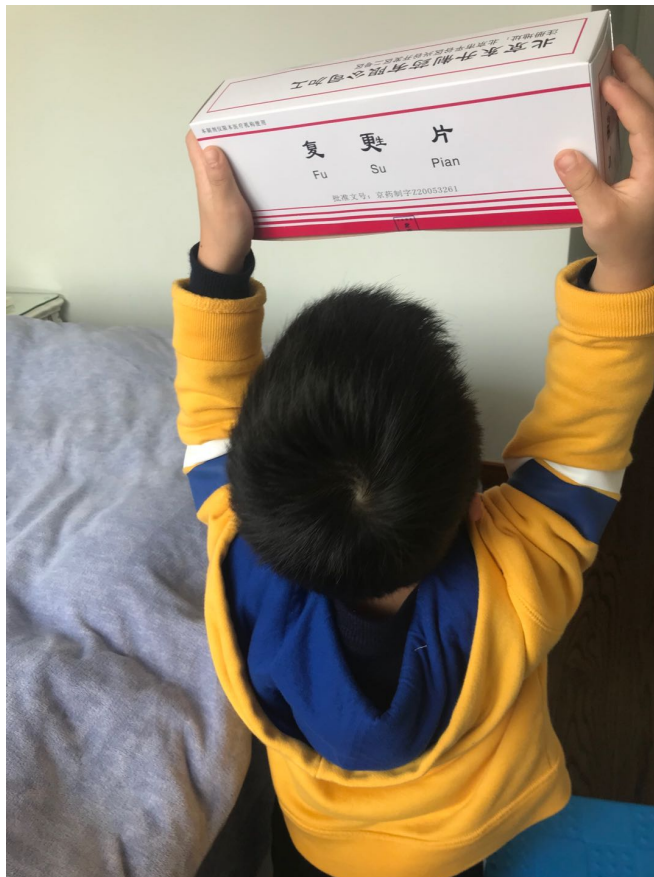
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Member of the Project's task force went to Peking Union Medical College Hospital to collect drugs for patients around the country in February 2020.



Medical professionals from the Peking Union Medical College Hospital checked drug inventory and details.



Patient received the medication.



Drugs were packed and ready for delivery.



病痛挑战基金会  
ILLNESS CHALLENGE  
FOUNDATION



In February 2020, when the pandemic was most severe in Wuhan, volunteers went to a pharmacy closed to the Wuhan Seafood Market to get drugs for patients.

*Report written by the Illness Challenge Foundation. Special thanks to Ms. LI Ling and Ms. YING Xiaoyu for translating this report into English.*

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