

Emma Green: Science Superstar



*Find out how Emma
decides to join a
CF clinical trial.*



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Clinical Trial Words to Know

Assent: Form that you will need to sign if you want to participate in the study. It explains the study in kid-friendly language.

Clinical trial: Another name for a research study.

Research protocol: Details of a study. It's like a recipe that you need to follow very closely.

Informed consent: Form that your parent(s) or legal guardian will need to sign that explains everything they need to know about you taking part in a research study. For example, it will explain things like why the study is being done, how long it lasts, and what specific things you will have to do.

Placebo: Pill or medication that looks just like the study drug but doesn't have any real medicine in it.

Principal investigator: The person in charge of the study which is typically the doctor.

Research coordinator: Person who works with the principal investigator and helps to run a study. This is the person you will likely see the most.

Study subject: Person (like you) who has given consent to take part in a research study.



Emma Green's room was a different kind of messy this afternoon. Along with the usual jumble of clothes and books, her floor was covered with instruction sheets, cardboard scraps, glue, and scotch tape. She and her best friend, Alex, were hard at work on their project for the upcoming sixth-grade science fair.

"I think this one could be our best submission yet," said Alex, admiring their creation. "I'm seeing a blue ribbon for us this year."

"Mmm-hmm," Emma mumbled, aimlessly cutting away at some scrap paper.

Alex looked at her. "What's wrong, Em?" she asked. "Don't tell me you're not loving our hovercraft."

"Sorry. The hovercraft is great. I'm just feeling a little nervous right now."

"But you're a science whiz. You could do this stuff in your sleep!"

"It's not that," said Emma. "There's this thing tomorrow, at the hospital where I get my CF care. I was asked to be part of a clinical trial."

"A trial? What did you do now? Do you need a lawyer?"

"Not that kind of trial, you goof! It means you try out new medicines to see how they'll work on people. They need volunteers, and it turns out I might be just the kind of person they're looking for. I'm supposed to go down there tomorrow to see if I'll do it."

“That sounds kind of cool,” said Alex. “So what’s up? Are you scared it will hurt?”

Emma shrugged. “I don’t think so, but ...” She picked up a plastic propeller and silently twirled it with her finger.

Alex frowned. “I’m sorry. I didn’t mean to upset you.” She gathered up a few pieces of small wood and evened them up in her hand. “Really, you’re the bravest person I know.”

“I’m not that brave.”



“Yes, you are!” said Alex. “I mean, just look at what you have to do every day with your treatments. On top of that, you do awesome in school, you’re friends with everybody, and you and your family organize a team for the CF Great Strides walk. Being in this trial is just the next step for you!”

“Yeah,” said Emma. “Maybe.”

“Yeah, maybe!” echoed Alex. She bounced a ball of crumpled paper off Emma’s head, laughing. Soon the two were racing around the room in a full-on paper snowball fight—until Emma’s mother opened the door.

“Hard at work, I see,” she said, smiling. “Just wanted to remind Emma it’s almost time for her treatments. Alex, will you be joining us for dinner?”

“Thanks, but I have to get home,” said Alex, stuffing her things into her backpack. She gave her friend a hug. “Good luck tomorrow, Emma!”

“Thanks. I’ll need it.”

For the next 45 minutes, Emma carefully completed her evening treatments: albuterol and hypertonic saline with the vest, followed by DNase, and lastly her inhaled antibiotic. *Sometimes I really get tired of doing all of this stuff*, she thought, gathering up her equipment.





That night at dinner, Emma was quieter than usual. Her little brother, Jack, took the spotlight for himself. “And that’s when Jeremy put the rest of the worm in his pocket! It was so funny!”

“Well,” said Dad, “at least you waited until we finished eating to tell us that one.” But he had spoken too soon. Mom was placing a big tray on the table.

“I hope everyone saved room for dessert. In honor of Emma’s big day tomorrow, I made her favorite—chocolate cake!”

“Sweet!” said Dad and Jack at the same time.

Dad clinked his glass with his knife. “Emma, I just want to say how proud we all are of you, for thinking about joining this clinical trial.”

“Hear, hear,” said Mom. “To our trailblazer.” All three clinked their glasses against Emma’s.

Emma could feel her face getting hot and her eyes burning. It was too much. She jumped up from her chair and ran down the hallway, slamming her bedroom door.

Jack broke the silence. “Can I have her piece?”

* * *

Mom knocked on Emma’s door and entered quietly. Emma was sobbing into her pillow, a stuffed bear under her arm. “Something you can talk about?” asked Mom.

“You and Dad and Alex are treating me like I’m some kind of hero,” said Emma, sniffing. “But I’m not. I’m really nervous about all this clinical trial stuff. I don’t even know if I want to do it.”

Mom pushed the hair off Emma’s forehead. “We’re always proud of you, honey.”



“But I get so tired of everything I have to do every day. The vest, all my medicines. Sometimes it feels like too much. And now they want me to add more?”

“You don’t have to do anything you don’t want to do,” said Mom. “We’ll support whatever you decide.”

She held Emma for a minute and patted her back. “Before bed, how about you and I look over the informed consent form they gave us? I think it gives a really good explanation of why they’re doing the clinical trial and what to expect if you decide to do it. Will that help?”

“Yeah, maybe,” said Emma. “Can I still have dessert?”



The next afternoon, Emma and her mother went straight from school to the CF center. Emma wondered if she would see Dr. Li, her favorite doctor ever.

In the waiting room, a friendly woman came out and extended her hand to Emma. "Hello! I'm Claire."

She looks friendly, Emma thought. And I like her hair.

"I'm happy you came to see me today," said Claire. "And you remembered to bring Mom!"

Emma laughed. *So far, so good.*

"Come on back and we can chat."

Claire led Emma to a clinic room decorated with brightly colored posters. "I'm the research coordinator for this clinical trial," said Claire. "You're going to meet a lot of other members of the research team if you do this, but I'm the most important one." They both laughed. "Really, it's my job to take care of our volunteers, so ask me anything, and I'll try to help."

"Will do," said Emma with a small smile.

"Now, this clinical trial will be what's called an interventional study," continued Claire. "That's when we ask volunteers to take new medicines, try new treatments, or do different things like using a new nebulizer. There's another kind of study, called observational, where we look at information, like height, weight, and breathing test results. With me so far?"

Emma and her mom nodded.

"Today is your screening visit. This is when we make sure you really *want* to be part of the study, and also that you're eligible to be in it. Dr. Li thinks you'll be a good fit, but we still have to look at your medical history and do some other tests, along with a physical exam. Do you have any questions for me?"

Emma spoke up. "How much more time will it take to do this ... interventional stuff?"

"She's a pretty busy girl," Mom explained.



“I know people with CF are busy. You work hard to fit in the treatments. We’re going to talk in just a bit about how this study might affect the care you do every day,” said Claire. “We’ll also go over why we’re doing the study, how many visits you’ll need, what we’ll do at your visits, and what you’ll need to do at home.”

“Sounds good,” said Emma.

“I want to make sure you know that you and your mom can ask me questions at any time. You can even call me, if you think of something later, OK? Now, I have a big question for you. What do you think is the most important thing here for all of us?”

Emma considered. “Seeing if the new medication works?”

“Good guess!” said Claire. “But what’s most important to us is *your* health. Dr. Li will be leading the research. So we’ll always talk with him and the rest of your care team to make sure you get the best possible care and to decide whether we should make any changes in your treatment plan.”

“Can she leave the study if she wants to?” asked Mom.

“Mom!” said Emma. “I’m fine!”

“You can leave the study at any time. Of course, we’d want to know why you changed your mind, like if it’s taking too much time or it makes you feel weird,” said Claire.

Emma chuckled. The more she learned, the more Emma warmed up to the idea of being in the study. *I can handle this*, she thought.

“If you do decide to participate in this study,” said Claire, “you’ll have to follow a protocol, which is like a recipe. If you don’t follow the recipe, you won’t get good results. Have you ever made pancakes, Emma?”

“I love pancakes!”

“Me too! What happens if you leave out the eggs?” Claire asked.

“They wouldn’t taste right,” said Emma.

“Right,” said Claire. “So we all have to follow the recipe.”

“So what’s in this recipe?” Emma asked. “Will it taste bad?” They all smiled.

“Really, though,” Emma said more seriously. “Will I get hurt? And what about my medications? Do I keep taking them?”

“Those are really good questions, Emma,” said Claire. “Yes, you have to have blood drawn at some of the visits. This will be just like the blood tests you get each year, and you know how that feels.”

That’s nothing, Emma thought.

“And you do need to keep taking all your other medications. As I said, what’s most important to all of us is your health,” said Claire.

Just then, a man carrying a laptop came into the research room. “I don’t believe you two need any introduction,” said Claire.

As Emma turned, her face lit up with recognition. “Dr. Li!”

“Emma,” said Dr. Li warmly. “I heard my friend was here. How are you feeling?”

“Fine,” said Emma, beaming. A lot of people asked her that question, but with Dr. Li, it felt like he really wanted to know. He had been Emma’s doctor for a long time, and he had been the one who helped Emma get the hang of giving herself her treatments.

“I hear you’re going to be part of this study, too!” said Emma.

“That’s right. I’ll be working with Claire and others to make sure everything is done correctly, so that whatever we learn from this study will work for as many people as possible. That also means we might do the same things over and over.”

“Thanks for the warning,” joked Emma.



“You know, I’m very proud of you for volunteering to be in this study,” said Dr. Li.

“I was pretty nervous at first, but now I want to do it. I hope I get in!”

“Me, too,” said Dr. Li. “What you’re doing is very important. Without kids like you, Emma, it would be hard to get any new CF medicines or get any closer to a cure.”

Somehow, it sounded more impressive to Emma coming from Dr. Li than it had from Dad.

“I’ve seen it happen,” Dr. Li continued. “The medication and treatment you take now got here because of people like you—people who were willing to help. By doing the trial, you can help the kids of the future. So thank you, from them.”

“You’re welcome, kids of the future,” said Emma, with a grin on her face.

For the next hour, Dr. Li gave Emma and her mom more details about the study. He told them about the risks and benefits of being in the trial—the good things and bad things that could happen. He reassured Emma that just because they were talking about them didn’t mean they were *going* to happen. Dr. Li just wanted Emma to know everything that might.

“Now, here comes the most popular question—again,” said Dr. Li. “Do you have any questions for us?”

Emma and her mom said no.

“OK,” said Claire, reaching for a folder. “Now that all the fun stuff’s out of the way,” she said, “how about some paperwork?”

Emma laughed. She felt safe with Claire. Together with Dr. Li, they read through a batch of papers. To Emma, they seemed a lot like school field trip permission slips. “This assent form is for you, Emma,” said Claire. “By signing this, you’re saying that you understand what you’re going to do.”

The other form was for Mom. It was the informed consent form she and Emma had looked at the night before. “Do we keep her regular clinic appointments?” asked Mom as she reviewed the form again.

“Absolutely,” replied Claire. “We do a good job talking with the clinic staff, and sometimes we can combine visits, but our study doesn’t replace Emma’s regular check-ups.”

As Mom signed the form, Emma felt butterflies in her stomach. She was getting excited to start!

Dr. Li had to sign the form as well. “We know it’s a lot to remember,” Claire said as they passed the form around. “So you’ll get a copy of the form, too, in case you have any questions after you get home.”

“Sounds good,” Emma said. Dr. Li gave her a quick hug, and headed out the door.

“Up next is a check-up just like you get during your CF clinic visits,” said Claire. “You’ll also go to the lab to get some blood drawn and give a urine sample. Then you come back over here to review the schedule. We want to make sure this study doesn’t clash with your plans.”

“I play soccer, and I’m on the debate team,” said Emma. “Will I still be able to do that stuff?”

“I told you she’s busy!” Mom said.

“I’m pretty sure we can make it work,” said Claire.

The check-up took a little longer than usual, but Claire was right—it was basically the same as her normal appointments. The whole time, Emma found herself eager to get back to Claire’s office to see if she could be in the study and if so, when she could start.

When they went over the schedule and dates for the study visit, Emma was happy to see that she’d only need to make a few extra visits. But she got quiet, and asked one last question. “If I’m not quite right for this study, would I get another chance?”

“Oh, yes,” said Claire. “If another study came up that you were just right for, we’d definitely bother you again.”

“You can bother me anytime!” said Emma.



The different kind of messy had returned to Emma's room. She and Alex were putting the finishing touches on their hovercraft and preparing for a test flight. Alex looked like she was about to burst.

"Well?" she asked. "How'd it go already?!"

Emma smiled. "The meeting went great—and I'm not nervous anymore. I should find more out soon. I need to have the right kind of blood, the right kind of health, and all that stuff. And I'll have to follow the recipe, or else the pancakes won't taste right."

Alex shot Emma a puzzled look, and then pegged her in the head with a paper ball.

The two friends went off on a laughing spree, the kind where it's hard to stop. And inside, Emma was happy about the choices she had made.



Did Emma get into the study? How did it all work out?

Emma got the good news the next day that she was eligible for the study. She enjoyed her clinical trial so much she went on to become one of the center's most active study volunteers. And her science fair hovercraft was a big success, too.

If you're curious about how clinical trials work, just ask your doctor and CF care team.

About the Cystic Fibrosis Foundation

The Cystic Fibrosis Foundation is the world's leader in the search for a cure for cystic fibrosis. The Foundation funds more CF research than any other organization, and nearly every CF drug available today was made possible because of Foundation support. Based in Bethesda, Md., the Foundation also supports and accredits a national care center network that has been recognized by the National Institutes of Health as a model of care for a chronic disease. The CF Foundation is a donor-supported nonprofit organization. For more information, please go to www.cff.org.



To learn about CF clinical research and the benefits of participating in a clinical trial:

- Visit www.cff.org/Find
- Call the CF Foundation's toll-free Clinical Trials Hotline
1-800-FIGHT-CF

The following websites also offer general information about clinical trials:

- The National Institutes of Health (NIH):
www.clinicaltrials.gov
- Hear what other children have to say about clinical trials:
www.nhlbi.nih.gov/childrenandclinicalstudies
- The U.S. Food and Drug Administration (FDA):
www.fda.gov/oashi/clinicaltrials



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