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# A GUIDE TO THE TREATMENT JOURNEY

For healthcare professionals

Please see Important Safety Information on pages 2-5 and accompanying full Prescribing Information for CASGEVY™.

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# INDICATION AND IMPORTANT SAFETY INFORMATION

## INDICATION

CASGEVY™ is indicated for the treatment of sickle cell disease (SCD) in patients 12 years and older with recurrent vaso-occlusive crises (VOCs).

## IMPORTANT SAFETY INFORMATION

### WARNINGS AND PRECAUTIONS

**Potential Neutrophil Engraftment Failure**

Neutrophil engraftment failure is a potential risk in hematopoietic stem cell (HSC) transplant, defined as not achieving neutrophil engraftment after CASGEVY infusion and requiring use of unmodified rescue $CD34^+$ cells. In the clinical trial, all treated patients achieved neutrophil engraftment and no patients received rescue $CD34^+$ cells.

Monitor absolute neutrophil counts (ANC) and manage infections according to standard guidelines and medical judgement. In the event of neutrophil engraftment failure, patients should be infused with rescue $CD34^+$ cells.

**Prolonged Time to Platelet Engraftment**

Longer median platelet engraftment times were observed with CASGEVY treatment compared to allogeneic HSC transplant. There is an increased risk of bleeding until platelet engraftment is achieved. In the clinical trial, there was no association observed between incidence of serious bleeding and time to platelet engraftment.

Monitor patients for bleeding according to standard guidelines and medical judgement. Conduct frequent platelet counts until platelet engraftment and platelet recovery are achieved. Perform blood cell count determination and other appropriate testing whenever clinical symptoms suggestive of bleeding arise.

**Important Safety Information continues on the following pages and please see accompanying full Prescribing Information for CASGEVY.**

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# IMPORTANT SAFETY INFORMATION (continued)

## WARNINGS AND PRECAUTIONS (continued)

### Hypersensitivity Reactions

Hypersensitivity reactions, including anaphylaxis, can occur due to dimethyl sulfoxide (DMSO) or dextran 40 in the cryopreservative solution. Monitor patients for hypersensitivity reactions during and after infusion.

### Off-Target Genome Editing Risk

Although off-target genome editing was not observed in the edited $CD34^+$ cells evaluated from healthy donors and patients, the risk of unintended, off-target editing in an individual's $CD34^+$ cells cannot be ruled out due to genetic variants. The clinical significance of potential off-target editing is unknown.

## ADVERSE REACTIONS

The most common Grade 3 or 4 non-laboratory adverse reactions (occurring in ≥ 25%) were mucositis, febrile neutropenia, and decreased appetite.

The most common Grade 3 or 4 laboratory abnormalities (occurring in ≥ 50%) include neutropenia, thrombocytopenia, leukopenia, anemia, and lymphopenia.

**Important Safety Information continues on the following pages and please see accompanying full Prescribing Information for CASGEVY™.**

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## IMPORTANT SAFETY INFORMATION (continued)

### DRUG INTERACTIONS

No formal drug interaction studies have been performed. CASGEVY™ is not expected to interact with the hepatic cytochrome P450 family of enzymes or drug transporters.

**Use of Granulocyte-Colony Stimulating Factor (G-CSF):** G-CSF must not be used for $CD34^+$ HSC mobilization of patients with SCD.

**Use of Hydroxyurea:** Discontinue the use of hydroxyurea at least 8 weeks prior to start of each mobilization cycle and conditioning. There is no experience of the use of hydroxyurea after CASGEVY infusion.

**Use of Voxelotor and Crizanlizumab:** Discontinue the use of voxelotor and crizanlizumab at least 8 weeks prior to start of mobilization and conditioning, as their interaction potential with mobilization and myeloablative conditioning agents is not known.

**Use of Iron Chelators:** Discontinue the use of iron chelators at least 7 days prior to initiation of myeloablative conditioning, due to potential interaction with the conditioning agent. Some iron chelators are myelosuppressive. If iron chelation is required, avoid the use of non-myelosuppressive iron chelators for at least 3 months and use of myelosuppressive iron chelators for at least 6 months after CASGEVY infusion. Phlebotomy can be used instead of iron chelation, when appropriate.

**Important Safety Information continues on the following pages and please see accompanying full Prescribing Information for CASGEVY.**

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## IMPORTANT SAFETY INFORMATION (continued)

### USE IN SPECIFIC POPULATIONS

**Pregnancy/Lactation:** CASGEVY™ must not be administered during pregnancy and breastfeeding should be discontinued during conditioning because of the risks associated with myeloablative conditioning. Pregnancy and breastfeeding after CASGEVY infusion should be discussed with the treating physician.

**Females and Males of Reproductive Potential:** A negative serum pregnancy test must be confirmed prior to the start of each mobilization cycle and reconfirmed prior to myeloablative conditioning.

Women of childbearing potential and men capable of fathering a child must use effective methods of contraception from start of mobilization through at least 6 months after administration of CASGEVY.

Infertility has been observed with myeloablative conditioning therefore, advise patients of fertility preservation options before treatment, if appropriate.

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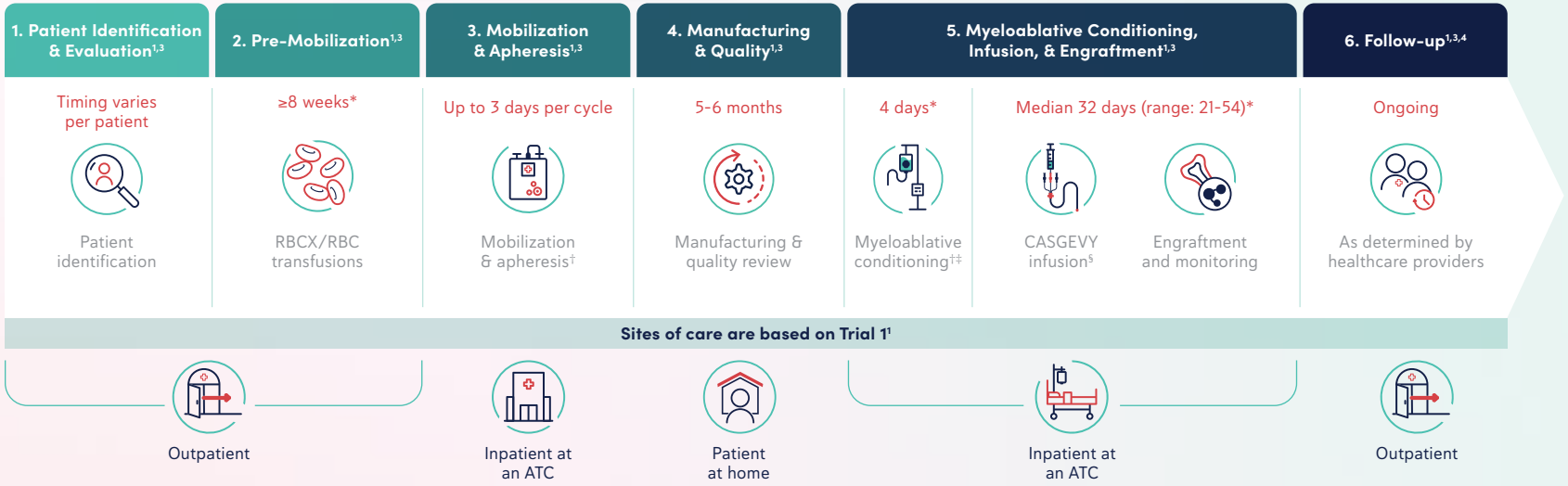
# Treatment Journey Overview

The time frame for each step of the CASGEVY™ treatment journey is approximate and will vary per patient. The entire CASGEVY treatment journey could take up to a year. Further details for each step can be found within the brochure. CASGEVY treatment is overseen by care teams at Authorized Treatment Centers (ATCs). Every ATC completed the onboarding process and associated training for CASGEVY. The ATC will determine which steps of the patient's care are inpatient or outpatient.[1,2] For a full list of ATCs near you and your practice, visit CASGEVYHCP.com.

If the minimum dose is not met after manufacturing, the patient will need to undergo additional cycles of mobilization and apheresis to obtain more cells. Each mobilization and apheresis cycle must be separated by a minimum of 14 days.[3]




RBC=red blood cell; RBCX=red blood cell exchange.
*Timing is based on Trial 1.[1,3]
†Discontinue disease modifying therapies for SCD 8 weeks before the planned start of mobilization and conditioning.[3]
‡It is recommended that patients be transfused at least 8 weeks prior to the initiation of myeloablative conditioning.[3]
§CASGEVY must be administered between 48 hours and 7 days after the last dose of myeloablative conditioning.[3]

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## Step 1:
## Patient Identification & Evaluation
**Timing varies per patient. Patient evaluation occurs at an ATC[1,3]**

**CASGEVY™ is indicated for patients 12 years and older with SCD with recurrent VOCs[3]**

Healthcare providers at an ATC will confirm which patients are appropriate for CASGEVY. The ATC will perform a benefits investigation to confirm the patient's insurance coverage and obtain prior authorization.

### Access and Reimbursement Education

The Vertex Access and Reimbursement Leads (ARLs) have knowledge of hospital administration and the payer reimbursement landscape. They are available to educate and address any questions ATCs have with respect to access and reimbursement for CASGEVY.

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## Order placement

Once a patient is determined to be eligible for CASGEVY™, the ATC will place an order through the Vertex Connects™ portal, a secure online order management system.[2]

## Conversations with your patients

- Discuss options if your patients have questions about insurance benefits and costs (refer to page 25 for additional information on the Vertex Patient Support Program)
- Patients should be prepared for extended periods of time away from home, work, or school at different points of the treatment journey[3]
- Talk to patients about having a personal support network, which can help them through the treatment process
- Advise patients of fertility preservation options before treatment, if appropriate. Myeloablative conditioning, required prior to CASGEVY, can cause infertility and be associated with other safety events[3]
- Women of childbearing potential and men capable of fathering a child must use effective methods of contraception from start of mobilization through at least 6 months after administration of CASGEVY[3]
- Encourage patients to ask questions throughout the process if there is something they do not understand or have concerns about

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## Step 2:
## Pre-Mobilization
**Minimum of 8 weeks on an outpatient basis[1,3]**

A healthcare provider at the ATC will determine the need for exchanges or transfusions.[2] RBC exchanges or simple transfusions are recommended prior to mobilization and apheresis.[2,3]

Screen patients for HIV-1, HIV-2, HBV, HCV, and any other infectious agents in accordance with local guidelines before collection of cells for manufacturing. CASGEVY™ should not be used in patients with active HIV-1, HIV-2, HBV, or HCV.[3]

The goal of RBC exchanges or transfusions is to maintain a hemoglobin S (HbS) level of <30% of total hemoglobin (Hb) while keeping total Hb concentration ≤11 g/dL.[3]

Discontinue disease modifying therapies for SCD (eg, hydroxyurea, crizanlizumab, voxelotor) 8 weeks before the planned start of mobilization.[3,5]



HBV=hepatitis B virus; HBC=hepatitis C virus; HIV=human immunodeficiency virus.

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**In Trial 1:**

- Patients underwent RBC exchanges or simple transfusions for a minimum of 8 weeks before the start of mobilization to reduce the number of circulating sickled cells and continued until initiation of myeloablative conditioning[2,6]
- RBC exchanges or simple transfusions were performed within 3 days of the start of mobilization to create a more stable interface for cell collection and decrease the risk of clotting and platelet aggregation, which can interfere with cell collection[2,5]

## Conversations with your patients

- Discuss the goals of RBC exchanges and transfusions with your patients prior to mobilization and apheresis to help set expectations before treatment begins[3]
- Review the transfusion process with your patients, including what type of vascular access device may be required, as they may be unfamiliar with the procedure or the reason for the device[2]

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**If the minimum dose is not met after manufacturing, the patient will need to undergo additional cycles of mobilization and apheresis to obtain more cells.[3]**

## Step 3:
## Mobilization & Apheresis
**Up to 3 days per cycle, inpatient at ATC[1,3]**

Patients are required to undergo mobilization followed by apheresis to isolate the CD34+ hematopoietic stem cells (HSCs) needed for CASGEVY™ manufacturing.[3]

### Mobilization

- Mobilization regimen increases yields of CD34+ HSCs prior to apheresis[7]
- Plerixafor was used for mobilization[3]

### Venous access for apheresis

The type of vascular access device–generally a central line placement–will be determined at the direction of the healthcare provider and the apheresis team at the ATC.[2,8]

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**In Trial 1:**

- Prior to the start of mobilization, patients were examined to ensure they were able to undergo mobilization and apheresis[6]
  - Examples of ineligibility in the clinical trial included patients with hemodynamic instability, positive infectious serologies, or active infection[6]
- Plerixafor was administered approximately 2-3 hours before the planned start of collection for up to 3 consecutive days[3]

**Keep in mind**

- Granulocyte-colony stimulating factor (G-CSF) should not be administered for mobilization in patients with SCD because it can induce severe VOCs[3,5]

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## Apheresis

Maximize CD34+ cell collection to obtain as many CD34+ cells as possible for CASGEVY™ manufacturing during each mobilization and apheresis cycle.[3]

### Days 1–2:

- Perform 2 consecutive days of cell collection for product manufacturing per cycle, if clinically tolerated. A total collection target of at least $20\times10^6$ CD34+ cells/kg is recommended for product manufacture[3]
- Collected cells to be used for the manufacture of CASGEVY are shipped each day from the ATC to the manufacturing facility. These cells should be sent for product manufacturing even if the total collection target is not achieved[2,3]

### Day 3:

- At least $2\times10^6$ CD34+ cells/kg is required to be collected for unmodified backup cells. A third day of cell collection can be used to obtain backup cells, if needed. If the target for backup cells is met, a repeat collection during any additional cycles will not be needed[2,3]

## Backup cells

Unmodified backup CD34+ HSCs may be used for rescue treatment under any one of the following conditions[3]:

- Compromise of CASGEVY after initiation of myeloablative conditioning and before CASGEVY infusion[3]
- Neutrophil engraftment failure[3]
- Loss of engraftment after infusion with CASGEVY[3]

## In Trial 1:

- All treated patients achieved neutrophil engraftment, and no patients received backup CD34+ HSCs[3]

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## Additional mobilization and apheresis cycles

- If the minimum dose of CASGEVY™ ($3\times10^6$ $CD34^+$ cells/kg) is not met after initial product manufacturing, the patient will need to undergo additional cycles of mobilization and apheresis[3]
  - A Vertex Care Manager will update the ATC care team if an additional mobilization and apheresis cycle is needed. This will happen approximately 3 weeks after the first collection[9]
  - Each mobilization and apheresis cycle must be separated by a minimum of 14 days from the first apheresis day of the prior cycle[3,5]

## Number of mobilization and apheresis cycles in Trial 1:

- The median number of cycles required was 2 (range: 1-6). Six (10%) patients were unable to receive CASGEVY therapy due to not achieving the minimum dose[3]

### Apheresis support

The Vertex Apheresis and Infusion Specialist (AIS) team can provide education and training to ATCs related to this step.[2]

## Conversations with your patients

- Patients and caregivers should be prepared for the impact on daily life associated with mobilization and apheresis, including time away from home and possible disruptions to work or school
- Discuss with patients and caregivers the possibility for multiple collection cycles[3]
- Remind patients to discontinue existing disease modifying treatments[3]
- Remind patients that a negative pregnancy test is required prior to the start of mobilization[3]

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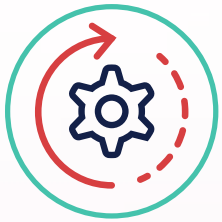

## Step 4:
## Manufacturing & Quality
**5-6 months at a manufacturing facility[1,3]**

During the CASGEVY™ manufacturing process, CRISPR/Cas9 technology is used to precisely edit HSCs at the erythroid-specific enhancer region of the *BCL11A* gene.[3]

### Manufacturing process

- Once the HSCs are received at the facility, it takes approximately 5-6 months to manufacture and quality test CASGEVY before it is sent back to the ATC[1,3]
- CASGEVY is shipped frozen in liquid nitrogen dry shippers to the ATC with delivery details and Global Positioning System (GPS) tracking information[2]

**Quality release testing**
After manufacturing CASGEVY, quality release testing is performed to confirm the product meets release criteria, including viability, purity, content, potency, sterility, and other safety release tests, before being shipped.[9]

### Keep in mind

- Although off-target genome editing was not observed in the edited CD34+ cells evaluated from healthy donors and patients, the risk of unintended, off-target editing in an individual's CD34+ cells cannot be ruled out due to genetic variants. The clinical significance of potential off-target editing is unknown[3]

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## Prior to myeloablative conditioning

- It is recommended that patients be transfused for at least 8 weeks with a goal to maintain HbS levels <30% of total Hb while keeping total Hb concentration ≤11 g/dL[3]
- Discontinue disease modifying therapies for SCD (eg, hydroxyurea, crizanlizumab, voxelotor) for at least 8 weeks[3]
- Iron chelation should be discontinued for at least 7 days[3]
- Prophylaxis for seizures should also be considered. Refer to the prescribing information of the conditioning agent used for information on drug interactions[3]

## Conversations with your patients

- Patients will not typically remain at the ATC during the manufacturing process. They usually return home until the next step of the process
- Patients will work with their ATC to schedule a time for their CASGEVY™ infusion

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## Step 5:
## Myeloablative Conditioning, Infusion, & Engraftment
**Approximately 6 weeks at an ATC based on the clinical trial (Trial 1)[1,3]**

### Myeloablative conditioning: 4 days[3]

- Myeloablative conditioning should only start once the availability of the complete set of vials comprising the total dose of CASGEVY™ and unmodified backup cells has been confirmed[3]

**In Trial 1:**

- Patients were admitted inpatient at the ATC and received 4 consecutive days of myeloablative conditioning with busulfan intravenously via a central venous catheter at a planned starting dose of 3.2 mg/kg/day once daily (qd) or 0.8 mg/kg every 6 hours (q6h)[1,3]
  - Busulfan plasma levels were measured by serial blood sampling and the dose adjusted to maintain exposure in the target range[3]
  - For once daily dosing, 4-day target cumulative busulfan exposure was 82 mg*h/L (range: 74 to 90 mg*h/L)[3]
  - For dosing every 6 hours, the 4-day target cumulative busulfan exposure was 74 mg*h/L (range: 59 to 89 mg*h/L)[3]
- Prophylaxis for hepatic veno-occlusive disease (VOD)/hepatic sinusoidal obstruction syndrome was administered, per regional and institutional guidelines[3]
- All patients received anti-seizure prophylaxis with agents other than phenytoin prior to initiating busulfan conditioning[3]

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## Infusion, engraftment, and monitoring: median 32 days in Trial 1 (range: 21–54 days)[1]

### Infusion

- Infusion of CASGEVY™ occurs in 1 day, at a minimum of 48 hours and a maximum of 7 days after the last dose of myeloablative conditioning[1,3]
- Patients are administered an antihistamine and antipyretic prior to the infusion[3]
- Each vial of CASGEVY should be thawed just prior to the scheduled infusion and infused within 20 minutes of thaw[3]
- Administer CASGEVY through a central venous catheter via IV push[3]
- Thaw and infuse one vial at a time; ensure all vials are administered[3]
- CASGEVY can cause hypersensitivity reactions due to dimethyl sulfoxide (DMSO) or dextran 40 in the cryopreservative solution. Monitor patients for hypersensitivity reactions during and after infusion[3]

## Infusion, engraftment, and monitoring: median 32 days in Trial 1 (range: 21-54 days)[1] (continued)

### Engraftment and monitoring

- After infusion of CASGEVY™, monitor for neutrophil and platelet engraftment. In the clinical trial, the median (min, max) time to neutrophil engraftment in patients (n=44) was 27 (15, 40) days. The median time to platelet engraftment in patients (n=43) was 35 (23, 126) days[3]
- Neutrophil engraftment failure is a potential risk in hematopoietic stem-cell transplantation (HSCT), defined as not achieving neutrophil engraftment after CASGEVY infusion and requiring use of unmodified backup CD34+ HSCs. In the clinical trial, all treated patients achieved neutrophil engraftment and no patients received backup CD34+ HSCs[3]
- Longer median platelet engraftment times were observed with CASGEVY treatment compared to allogeneic HSCT. There is an increased risk of bleeding until platelet engraftment is achieved. In the clinical trial, there was no association observed between incidence of serious bleeding and time to platelet engraftment[3]

### Keep in mind

- Monitor absolute neutrophil counts (ANC) and manage infections according to standard guidelines and medical judgment. In the event of neutrophil engraftment failure, patients should be infused with backup CD34+ HSCs[3]
- Monitor patients for bleeding according to standard guidelines and medical judgement[3]
- Conduct frequent platelet counts until platelet engraftment and platelet recovery are achieved. Perform blood cell count determination and other appropriate testing whenever clinical symptoms suggestive of bleeding arise[3]

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## Monitoring and prophylaxis in Trial 1:

- Patients underwent infectious surveillance and prophylaxis (bacterial, viral, and fungal) following the infusion of CASGEVY™. Patients were monitored and medically managed if any signs or symptoms of sepsis or systemic bacteremia occurred[6]

## Conversations with your patients

- Advise patients of the risks associated with mobilization and myeloablative conditioning agents and to read the FDA-approved patient labeling (Patient Information) for these agents[3]
- Fertility preservation is an important consideration for patients to discuss with their healthcare provider before deciding on gene therapy. Myeloablative conditioning, required prior to CASGEVY, can cause infertility and be associated with other safety events. Patients pursuing fertility preservation need to complete the process prior to myeloablative conditioning[3]
- A negative serum pregnancy test must be confirmed prior to the start of each mobilization cycle and reconfirmed prior to myeloablative conditioning[3]
- After myeloablative conditioning and receiving CASGEVY, patients should be prepared for several weeks of inpatient recovery at the ATC[3]

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## Step 6:
## Follow-up
**Ongoing**[1,3,4]

Patients who received CASGEVY™ will have follow-ups post treatment, as determined by their healthcare providers.[3]

### After CASGEVY administration

Standard procedures for patient management after HSCT should be followed[3]:

- Irradiate any blood products required within the first 3 months[3]
- Patients should not donate blood, organs, tissues, or cells at any time in the future[3]
- Restarting iron chelation may be necessary. Avoid the use of non-myelosuppressive iron chelators for at least 3 months and the use of myelosuppressive iron chelators for at least 6 months after CASGEVY infusion. Phlebotomy can be used in lieu of iron chelation, when appropriate[3]

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**In Trial 1:**

- Following engraftment, RBC transfusions were avoided for patients with Hb levels ≥7 g/dL, unless medically indicated (eg, symptomatic anemia or as a requirement for surgery)[6]

## Conversations with your patients

- Your patients may have questions about the treatment journey and their follow-up plan post treatment. Be sure to walk them through what will be required at each milestone so they have a good grasp of what to expect with CASGEVY™

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# A Guide to Effective Conversations With Your Patients

## Step 1: Patient Identification & Evaluation

- Discuss options if your patients have questions about insurance benefits and costs (refer to page 25 for additional information on the Vertex Patient Support Program)
- Patients should be prepared for extended periods of time away from home, work, or school at different points of the treatment journey
- Talk to patients about having a personal support network, which can help them through the treatment process
- Advise patients of fertility preservation options before treatment, if appropriate. Myeloablative conditioning, required prior to CASGEVY™, can cause infertility and be associated with other safety events
- Women of childbearing potential and men capable of fathering a child must use effective method of contraception from start of mobilization through at least 6 months after administration of CASGEVY
- Encourage patients to ask questions throughout the process if there is something they do not understand or have concerns about

## Step 2: Pre-Mobilization

- Discuss the goals of RBC exchanges and transfusions with your patients prior to mobilization and apheresis to help set expectations before treatment begins
- Review the transfusion process with your patients, including what type of vascular access device may be required, as they may be unfamiliar with the procedure or the reason for the device

## Step 3: Mobilization & Apheresis

- Patients and caregivers should be prepared for the impact on daily life associated with mobilization and apheresis, including time away from home and possible disruptions to work or school
- Discuss with patients and caregivers the possibility for multiple collection cycles
- Remind patients to discontinue existing disease modifying treatments
- Remind patients that a negative pregnancy test is required prior to the start of mobilization

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# A Guide to Effective Conversations With Your Patients

## Step 4: Manufacturing & Quality

- Patients will not typically remain at the ATC during the manufacturing process. They usually return home until the next step of the process
- Patients will work with their ATC to schedule a time for their CASGEVY™ infusion

## Step 5: Myeloablative Conditioning, Infusion & Engraftment

- Advise patients of the risks associated with mobilization and myeloablative conditioning agents and to read the FDA-approved patient labeling (Patient Information) for these agents
- Fertility preservation is an important consideration for patients to discuss with their healthcare provider before deciding on gene therapy. Myeloablative conditioning, required prior to CASGEVY, can cause infertility and be associated with other safety events. Patients pursuing fertility preservation need to complete the process prior to myeloablative conditioning
- Having a personal support network will help patients through the treatment process
- After myeloablative conditioning and receiving CASGEVY, patients should be prepared for weeks of inpatient recovery at the ATC

## Step 6: Follow-up

- Your patients may have questions about the treatment journey and their follow-up plan post treatment. Be sure to walk them through what will be required at each milestone so they have a good grasp of what to expect with CASGEVY

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VERTEX Connects™

## Vertex Connects

Vertex Connects is a program that provides educational resources, communications, and support to navigate the treatment journey for patients and their loved ones who have been prescribed a Vertex gene therapy.

## Vertex Connects offers support through Care Managers who will:

- Share educational resources to help patients prepare for each step
- Provide information and help answer questions along the treatment journey
- Work with Authorized Treatment Centers to help coordinate logistics of each patient's treatment journey

Confirmation of eligibility and a completed enrollment form are required to enroll in Vertex Connects Patient Support. Enrollment in Vertex Connects Patient Support is not required for receiving CASGEVY™.

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**For more information about CASGEVY™ including clinical trial results, safety, MOA, and additional resources, visit CASGEVYHCP.com.**

**Please see Important Safety Information on pages 2-5 and accompanying full Prescribing Information for CASGEVY.**

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